Professional MBA
Entrepreneurship & Innovation

InvolveMe-Business plan, closing gaps in translational research by transiting patients from drug consumers to participants in research

A Master's Thesis submitted for the degree of
"Master of Business Administration"

supervised by
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Vienna, 01.07.2016

Affidavit

I, **Dr. Rana Grillberger**, hereby declare

1. that I am the sole author of the present Master's Thesis, "InvolveMe-Business plan, closing gaps in translational research by transiting patients from drug consumers to participants in research", 91 pages, bound, and that I have not used any source or tool other than those referenced or any other illicit aid or tool, and
2. that I have not prior to this date submitted this Master's Thesis as an examination paper in any form in Austria or abroad.

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Signature

ABSTRACT

The master thesis "**InvolveMe-Business plan, closing gaps in translational research by transiting patients from drug consumers to participants in research**" demonstrates how a service provider can create in a profitable manner the conditions of enrichment of biomedical science produced in labs through patients´ involvement.

Despite being the primary beneficiaries of translational research, the patients´ voice is not included in the innovation process in the design of new lab research. InvoveMe is a novel patients- and researchers- oriented service venture that enables the communication of jargon free biomedical and pharmaceutical knowledge by researchers to patients with rare diseases through crowdsourcing platforms and conferences. InvolveMe invites also patients affected by a specific rare disease to submit questions that researchers researching the same disease may be able to answer. Thereby it enables the injection of the patients´ unique experiential knowledge in the translation process of basic scientific findings to relevant clinical applications.

Striving for being the preferred bridge between patients with rare diseases and biomedical researchers from the public and private sector, InvolveMe partners with key patients´ support organizations that are representative of people living with rare diseases in the world and communicates its services to researchers by back-riding on sales and marketing channels of related non-competing businesses for a fast and efficient market entry.

Despite being entirely dependent on researchers and patients in the creation of its services, InvolveMe enables research organizations for rare diseases to coincide their biomedical innovation model with the concerns of their end users.

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1 INTRODUCTION

Despite being the original object of concern in biomedical research and the future users of drugs, patients do not play an active role in translational research. Translational research is defined as the 'translation' process of basic scientific findings to relevant and useful clinical applications. Until today, the biomedical research process follows a linear innovation model. The innovation chain begins with new approaches and methods in lab followed by animal studies which are subsequently tested in studies with humans ultimately leading to health gains for the individual patient. Patients hardly emerge in this view except in their role as passive research subjects. To add insult to injury, many scientific results never reach the clinic, leading to concerns that scientific research does not necessarily produce the anticipated benefits. As an example, only 5 % of the target discoveries result in approved new drugs suggesting that the disappointing results are due to gaps in the biomedical innovation process. Hence, incorporating insights from patients' considerations into lab research is one strategy that may help achieve the aim of translational research.

This master thesis describes a new for-profit service venture named InvolveMe whose mission is to trigger a discourse between researchers of therapeutic solutions for rare diseases and patients affected by the same disease researched. InvolveMe helps on one side curious patients to get informed directly from researchers through conferences held in a jargon free manner about scientific innovations concerning their own diseases and offer on the other side life sciences organizations access to a list of unanswered patients´ questions that research may answer. However, while patients would be delighted by InvolveMe services, InvolveMe has to find motives for researchers from the private and public sector to use its services especially that researchers often do not experience a sense of urgency to involve patients in research. Furthermore, as a new conference organizer in the field of rare diseases, InvolveMe has to differentiate itself from the competition.

To attract patients and researchers, InvolveMe has to deliver convenient services with significant benefit to them. Additionally, to survive, InvolveMe has to employ strong

marketing tools aiming to communicate its offerings and increase participation and attendance. Finally, InvolveMe has to know how to design its new services in a manner that fulfills and satisfies customers´ requirements. Therefore, the following research questions were raised:

- What are the known characteristics of services in general and how to set characteristics for InvolveMe services to improve the customer experience when using them?
- What marketing concepts should InvolveMe formulate as a service provider to communicate its offerings and increase attendance to its conferences?
- Finally, how should InvolveMe design its new services in a way that delights customers?

The thesis covers in section 2 a literature research regarding the raised questions. The rest of the thesis proceeds as follows: section 3 presents the business plan of InvolveMe where the business model, the industry, the technology, the marketing plan, the sources of financing and the organizational structure are described. In section 4, the results of a survey done among researchers and patients are shown. Finally, Section 5 features concise answers to the research questions rose and include few proposals for future research.

2 THEORETICAL FRAME

2.1 Services characteristics

Research has considered that services differ from products in terms of four key characteristics such as, intangibility, heterogeneity, inseparability and perishability (IHIP)[1].

Intangibility. Services are not physical objects. A humorous illustration describes services as "something that one cannot drop on his/her foot"[2]. Customers assess in general services based on past experience, word of mouth, or even the location and decor of the service venture. Thus, service providers must try to make as much as possible their intangible services tangible.

Heterogeneity. A service appears to be heterogeneous, due to the relative inability of standardizing its output in contrast to goods and to the inclusion of a large measure of the "human element". Hence, with many services, customers are purchasing nothing else but the skills of the suppliers.

Inseparability. The production of a service cannot be separated of its consumption. Accordingly, a service provider could not provide his offering without participation of the customer. Such challenge has implications both for channels of distribution and scale of operations. Thus, a service provider should minimize the inseparability issue by separating the service provided from the customer.

Perishability. Services cannot be stored in an inventory like a good. Perishability refers to the inability of services to be stored, resold, saved or returned. Therefore, service providers should consider making their services inventoried.

2.2 Understanding of marketing and its role in a venture

Marketing is the process of voluntarily exchanging products or services between

companies and customers so that both parties are satisfied. A venture must innovate to provide products and or services that will meet today's consumers' needs and desires, and will contribute to their maximum satisfaction. In doing so, ventures need to deeply investigate changes in the environment (domestic and international) and among target customers, and apply as is indicated in Figure 1 an appropriate combination of the service marketing mix elements to ensure high profitability in the long run.

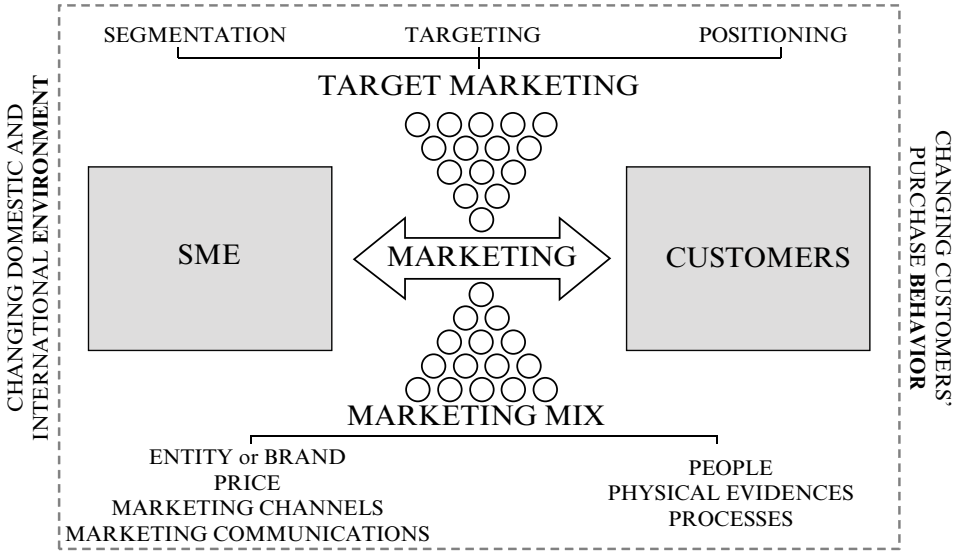


Figure 1. Defining a new venture marketing and its core concepts

Source: Ruzzier et al., 2013[3]

Target marketing and the marketing mix are the two marketing concepts of a marketing strategy. Both are conducted prior to the launch of the product/service in the market, which ultimately reaches the target customer through advertising, sales and promotion strategies and tactics.

2.2.1 Target marketing

Target marketing consists of three specific steps: segmentation, targeting and positioning.

2.2.1.1 Segmentation

Segmentation refers to the division of the market to specific groups of consumers who have similar wants and needs. These consumer groups are named segments.
The most important segmentation approaches are geographic, demographic, psychographic, behavioral and benefit segmentation[(4)].

Geographic segmentation. Geographic segmentation divides the local or global market into geographic subsets, such as countries or regions.

Demographic segmentation. Demographic segmentation is widely used and based on readily available and measurable consumer characteristics such as age, gender, occupation, education, disease etc. In business-to-business marketing, conceptually equivalent measures are company age, size, and profitability, as well as the type of industry.

Psychographic segmentation. Psychographic segmentation uses attitudes, interests, opinions, values and lifestyle to explain differences between consumers' behavior.

Behavioral segmentation. Behavioral segmentation focuses on whether costumers pay for a product or service and use it.

Benefit segmentation. Global benefit segmentation focuses on the benefit or value consumers try to achieve.

In the whole, there are various routes to segment a market and numerous market segments can be created. However, the most important issue is to identify the appropriate segment to strive toward the business goals.

2.2.1.2 Targeting

Targeting refers to evaluating and comparing the identified groups and then selecting

one or more of them with the highest potential. The market segment chosen is called the target market. The choice of the target market should generally depend on several factors. The first one is to select a segment whose needs are not currently being served well. Secondly, the size of the segment and its expected future growth keeping in mind that rapidly growing segment tend to attract competition.

2.2.1.3 Positioning

For each chosen target market, the new venture has to develop a market offering and position this in the minds of the potential customers. Positioning is the location of a service/product in the consumer's mind as a name and an idea relative to competing products.

2.2.2 Service marketing mix

The place to start is to ask what elements constitute a marketing strategy. As the basic elements constituting marketing consists of a marketing mix that is product or service, price, promotion, place (distribution) according to the four Ps introduced by McCarthy in 1960[(5)], it is appropriate to use this concept in a first step. However, for service, it was observed that the traditional marketing mix was inadequate because the 4Ps were developed for manufacturing industries. Therefore, the marketing mix has extended beyond the 4Ps for marketing of services. The three additional Ps are people, physical evidence, and process as shown in Figure 2.

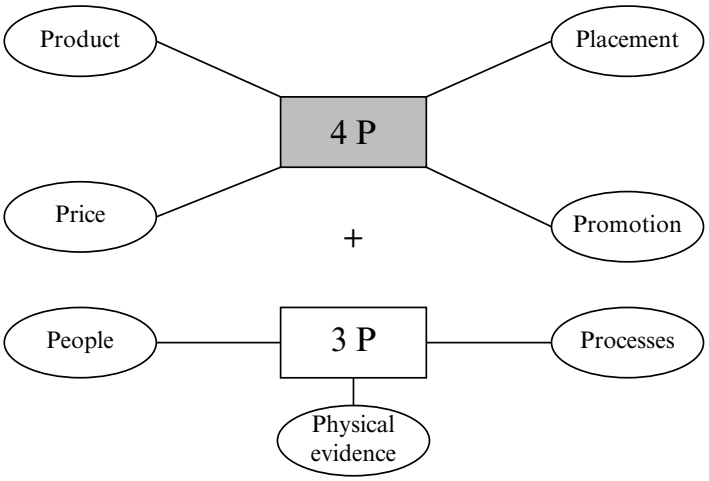


Figure 2. Service marketing mix.

Source: Ruzzier et al., 2013[3]

2.2.2.1 Product

This element indicates a description of the service to be marketed in the new venture. However, as previously mentioned, services are intangible, heterogeneous, perishable and their production and consumption by the customers are inseparable. Thus, because of the nil or minimal tangible component, the service offering should describe the bundle of features and benefits that make the service distinctive from many other existing competitors. While developing a service product it is therefore important that the package of benefits in the service offer has a customer's perspective.

2.2.2.2 Pricing

Price can be considered as an attribute that must be scarified to obtain certain kinds of services[6]. The service pricing must provide value addition and quality indication to the customers[7]. Therefore, a service provider must not only set prices that targeted customers are willing to pay, but also convey the message that customers are getting more in using the service offered.

Under the pricing of the 7Ps, entrepreneurs need to consider three important elements prior to setting the price. These elements are: costs, margins or markups, and competition[8].

Costs. The entrepreneur must first ascertain the costs that are directly related to the product or service. The costs for a service venture relate to the costs of labor and overhead expenses. The cost of labor refers to the sum of all wages paid to employees, as well as the cost of employee benefits and payroll taxes paid by an employer. Overhead refers to all ongoing business expenses not including or related to direct labor. A service-based business that operates in a traditional office setting would have overhead expenses such as rent, utilities and insurance.

Margins or Markups. Once costs are determined, the entrepreneur needs to mark up the service to ensure that he/she achieves a profit for her business. The objective is to achieve a desirable profit margin without getting a reputation for overcharging for services. To determine whether the profit margin is on target, it is recommended to look for resources in the same industry.

Competition. An entrepreneur must be aware of what competitors are charging for similar services in the marketplace. This information could come from competitor websites or talking to friends who have used a competitor's service. To justify a higher price than that of a competitor, the entrepreneur must provide a service with unique benefits that set his/her apart. By this, the entrepreneur is able to convince customers that they get a tremendous value in terms of service and quality. A higher price maybe supported by market research data. For instance, marketing research may reveal that customers are willing to pay more if the entrepreneur offers service benefits. Although these services would increase the costs to the entrepreneur, they would establish a distinctive image for the product in a non differentiated service category, allowing a higher price and, a higher quality image than that of the competitors.

2.2.2.3 Place

It is not possible to separate services from selling; they must be created and sold at

the same time[9]. Since service delivery is concurrent with its production and cannot be stored or transported, traditional distribution channels available for product marketing cannot be used in services marketing.

2.2.2.4 Promotion

It is usually necessary for the entrepreneur to inform potential consumers about the product/service availability or to educate the consumer. Promotion represents the communications that marketers use in the marketplace including advertising, public relations, personal selling and sales promotion[5]. In a new venture it is not possible to use the conventional promotion tools with success due the heavy promotional budget required. Therefore, promotional activities like social media and communities´ relations have relevance and can be used effectively with low budget to induce people to order services.

2.2.2.5 People

The intangible nature of services resulted in the addition of a further element that is 'P', People[10]. Judd´s argument was that it is the employees of an organization who represent the organization to the customers. Therefore, people are a defining factor in a service delivery process as a service is inseparable from the person providing it. The way a service is delivered by the people can be an important source of differentiation as well as competitive advantage. Here, it can be concluded that employees of InvolveMe should be trained to show personal attention to their customers in order to contribute toward the strength of customer-employee relationship.

2.2.2.6 Physical evidence

Services are often intangible, and customers cannot assess their quality well. Service environments, also called physical evidence, relate to the style and appearance of the physical surroundings and other experiential elements encountered by customers at service delivery sites[11]. Service firms need to manage physical evidence carefully by incorporating certain tangible elements into their offering as it can have a profound

impact on customers' impressions.

2.2.2.7 Process

Process describes the method and sequence in services and creates the value proposition that has been promised to customers. The process of service delivery is crucial as it ensures that the same standard of service is repeatedly delivered to the customers. A well designed process assures service availability, consistent quality, total ease and convenience to the customers[12]. As a conclusion, a service provider has to use simplified and user friendly processes.

2.3 Customer Satisfaction, Dissatisfaction and Delight

A new venture will achieve success if it creates value to customers, which is translated into customer satisfaction. This value represents the consumers' perception of how a particular product fulfills his or her needs and wants. Value creation may be defined as the ratio between what the customer gets (benefits) and what they give for it (costs). Hence, it is imperative for the service provider to consider the targeted customer base and their needs and expectations. This will help in developing a product/service design that will help the provider to effectively manage customer expectations leading to customer delight.

So far customer satisfaction was mostly seen as the higher the perceived product/service quality, the higher the customer's satisfaction and vice versa. But fulfilling the individual product/service requirements to a great extent does not necessarily imply a high level of customer satisfaction. Departing from Kano's model of customer satisfaction, the methodology is introduced to determine which influence the components of services have on customer satisfaction. To identify and prioritize service features that satisfy customers, the Kano model can be employed.

Kano Model Concept. In the past 30 years the Kano model has been used to prioritize product features. This model is a valuable tool to quantify requirements of a service

from a user-centered perspective. In his model, Kano provides a graphical representation of the relationship between three type of product attributes and customer satisfaction or dissatisfaction[13] (Figure 3).

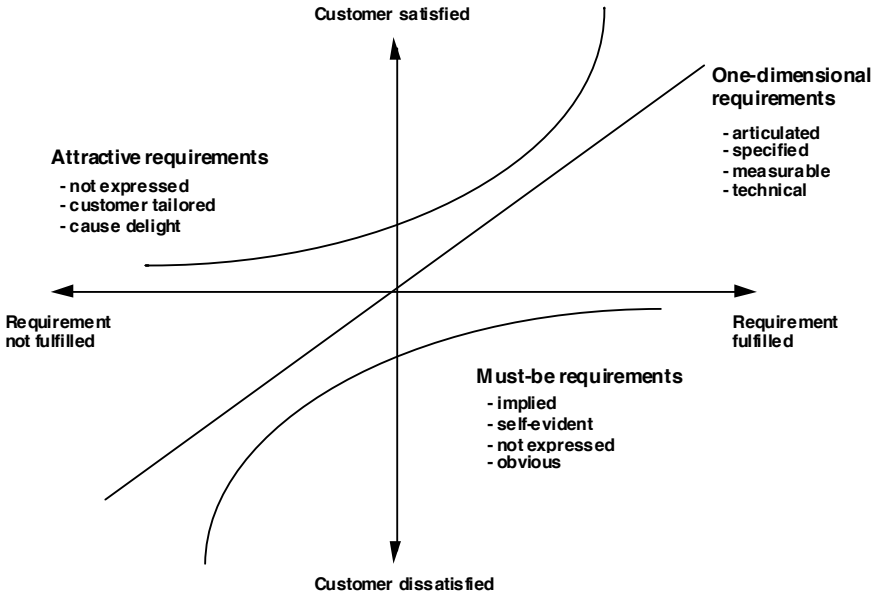


Figure 3. Kano Model of customer satisfaction

Source Berger et al., 1993[14]

Type of product/service requirements. Kano distinguishes between three types of product (or service) requirements which influence customer satisfaction in different ways when met:

Must-be quality requirements. The absence of must-be requirements is exponentially related to dissatisfaction meaning that the customer will be extremely dissatisfied. Because the customer takes these requirements for granted, their fulfillment will not increase his satisfaction. The must-be requirements are basic criteria of a product/service for the customer to be satisfied at all. Fulfilling the must-be requirements does not increase customer satisfaction and will only lead to a state of "not dissatisfied". The customer regards the must-be requirements as prerequisites, he takes them for granted and therefore does not explicitly voice them.

One-dimensional requirements. One-dimensional requirements are linearly related to customer satisfaction or dissatisfaction: the higher the level of fulfillment, the higher the customer's satisfaction and vice versa. One-dimensional requirements are usually known and explicitly voiced by the customer.

Attractive requirements. These requirements exhibit an exceptional relationship with satisfaction. When they lack, the customer is not dissatisfied but the presence of the requirement with a given service leads to an extremely favorable reaction, delighting the customer and providing the "wow" factor in the product/service usage experience. Therefore, fulfilling these requirements leads to more than proportional satisfaction. Often, attractive requirements are neither articulated nor expected by the customer, and hence they must be discovered. If the requirements are not met, however, the customer is not dissatisfied.

In the following, I explain how products/service requirements are ascertained by means of a questionnaire and how the results are evaluated and interpreted to design product/services. The objective of these questions is to design services that fulfill all basic needs, characterizing the customer requirements on one-dimensional requirements and strongly contribute to customer satisfaction to stand out with delighting requirements from competitors.

Construction of the Kano questionnaire. Customers should be asked two questions for each service/product requirement. For each service feature a pair of questions is formulated to which the customer can answer in one of five different ways[13]. The first question concerns the reaction of the customer if the service has that feature (functional form of the question), the second concerns his reaction if the service does not have that feature (dysfunctional form of the question). An example is shown below.

Example of a functional question in the Kano questionnaire. What would you think if you would get the possibility to share with patients during a conference your recent findings in a jargon free manner regarding a potential therapeutic solution they are waiting for?

- I like it that way
- It must be that way

- I am neutral
- I can live with it that way
- I dislike it that way

Example of a dysfunctional question in the Kano questionnaire. What would you think if you are not able to share with patients during a conference your recent findings in a jargon free manner regarding a potential therapeutic solution they are waiting for?

- I like it that way
- It must be that way
- I am neutral
- I can live with it that way
- I dislike it that way

Kano evaluation table. The answers to both questions are classified into 6 different requirements according to the matrix in Table 1.

Table 1. Kano evaluation Table

Customer requirement*		**Dysfunctional (negative) question**				
		Like	**Must-be**	**Neutral**	**Live with**	**Dislike**
Functional (positive) question	**Like**	Q	A	A	A	O
	Must-be	R	I	I	I	M
	Neutral	R	I	I	I	M
	Live with	R	I	I	I	M
	Dislike	R	R	R	R	Q

*Customer requirement is: A: Attractive; M: Must-be; R: Reverse; O: one-dimensional; Q: Questionable; I: Indifferent.

Attractive (or delighter criteria). Attractive are criteria which have the greatest influence on how satisfied a user will be with a given service. According to Table 1, if the customer answers, for example, "I like it that way," to the functional form of the question, and answers "I am neutral," to the dysfunctional form of the question, the combination of the questions in the evaluation table produces category A, indicating that being able to share with patients the recent findings regarding a therapeutic solution is an attractive customer requirement from the customer's viewpoint.

Must-be (or basic needs). If basic needs are not fulfilled, the user is extremely dissatisfied. (see section "Kano model concept and type of products requirements").

One dimensional. With one-dimensional requirements, user satisfaction is proportional to the level of fulfillment.

Indifferent. If combining the answers yields category I, this means that the customer is indifferent to this service feature and does not care whether it is present or not. He is, however, not willing to spend more on this feature.

Questionable. Category Q stands for questionable result. Normally, the answers do not fall into this category. Questionable scores signify that the question was phrased incorrectly, or that the person interviewed misunderstood the question or crossed out a wrong answer by mistake.

Reverse. If looking up the answer in the evaluation table yields category R, this service feature is not only not wanted by the customer but he even expects the reverse. For instance, when going to a conference, a company does not want to meet patients. Such evaluation can help identifying a specific customer segment who wants to share research results with patients while another would dislike it.

In addition to the Kano questionnaire, it might be helpful to have the customer rank the individual feature of the service and to determine the relative importance of individual service features based on self-stated-importance. This will help the entrepreneur establish priorities for service development and make improvements wherever necessary. An example of such questions could be: how would you rank from unsatisfactory (1) to excellent (7) the jargon free communication of research findings between your institution and patients in general? Answers can be scored from not at all important (1) up to extremely important (7).

Evaluation and Interpretation. The evaluation of the answers is performed according to frequency and customer satisfaction coefficients. The results of the questionnaire are analyzed by frequency, the highest frequency defines which category (Attractive, One-Dimensional, Must-be or Indifferent) the service requirement falls into. The highest frequency defines which category the service requirement falls into. For example, in Table 2 we can see that “Connection to patients” was categorized as

attractive by 60 percent of the respondents, 'Price' as a "must-be need" by 40%.

Table 2. Example of frequency and customer satisfaction coefficient

Service feature	A	O	M	I	Category	Extent of satisfaction*	Extent of dissatisfaction**
Connection to patients	60	10	30	0	Attractive	0.70	-0.40
Price	15	35	40	10	Must-be	0.50	-0.75

* Extent of satisfaction= (A+O)/(A+O+M+I)

** Extent of dissatisfaction=(O+M)/-(A+O+M+I)

To calculate the average extent of satisfaction it is necessary to add the Attractive (A) and one-dimensional (O) columns and divide by the total number of Attractive (A), One-dimensional (O), Must-be needs (M), and indifferent (I) responses; (A+O) / (A+O+M+I) (Table 2). For the calculation of the average "Extent of dissatisfaction" the must-be Needs (O) and One-Dimensional (O) columns must be added and then sum is then divided by the same factor; (O+M)/-(A+O+M+I). The average "Extent of satisfaction" and "dissatisfaction" measures show whether satisfaction can be increased by meeting a service requirement, or whether fulfilling this requirement merely prevents the user from being dissatisfied. The "Extent of satisfaction" ranges from 0 to 1. When the value is to 1, the influence on user satisfaction is higher. The "Extent of dissatisfaction" ranges from 0 to -1. The closer the value to -1, the more significant influence of the service feature on dissatisfaction. Figure 4 gives an example of how the results can be displayed when using the results shown in Table 2. Figure 4 shows that offering connection to patients is likely to delight customers and contribute to excitement and satisfaction.

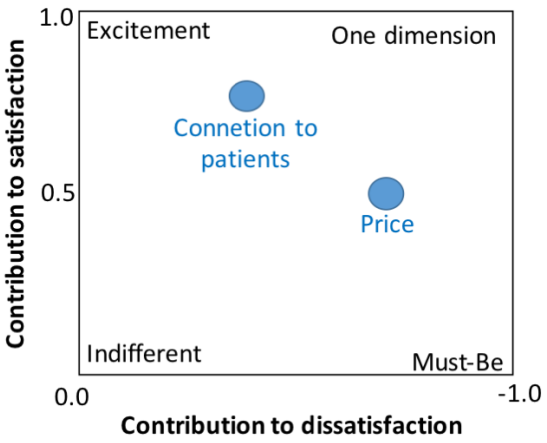


Figure 4. Results interpretation - Kano method

As a conclusion, If InvoveMe knows to what extent a service feature influences the perceived service quality by its customers according to Kano categories (must-be, one-dimensional or attractive requirement) the satisfaction portfolio of customers can be defined in order to establish a strong competitive advantage.

3 BUSINESS PLAN

3.1 Executive Summary

Introduction. Every year, more than 100 medicines are registered as medicines in development for rare diseases, so called orphan drugs. The main reason is that the development of orphan drugs is financially incentivised by law. However, only 5 % of medicines undergoing a clinical trial process proof to be safe, efficient and legal to market. These disappointing results are very likely due to gaps and bottlenecks in the biomedical innovation process. One of these gaps may be remedied by the incorporation of insights from patients' considerations into lab research.

Unique selling proposition. Recognizing this gap, InvolveMe is the first patient- and researchers oriented service venture founded for the purpose of connecting patients with rare diseases to biomedical researchers researching the same disease. Through the crowdsourcing platforms and conferences organized by InvolveMe, researchers communicate to patients their latest innovations in a jargon free manner. In turn, patients raise unanswered questions to researchers through the crowdsourcing platforms of InvolveMe and seek their answers. Thereby, researchers answer the patients questions while formulating new research questions that coincide with projected patients´ concerns.

Market Trend. Since 2000, more than 1000 orphan drugs have been under a clinical trial process for various indications according to the European register of all designated orphan medicinal products. Today, rare diseases receive significant attention from the European Commission who initiated together with national and international partners the international rare diseases research consortium that has a key objective to deliver by 2020 200 new therapies for rare diseases. Finally, it was reported that the market for orphan drugs is expected to grow by 11.7% per year from $114bn in 2016 to $178bn in 2020. These projections reflect the large number of research and development organizations initiating and financing clinical trials for medicines for rare diseases.

Management and characteristics. InvolveMe is a for profit service venture that is foreseen to be founded by Rana Grillberger and another partner that is not determined yet. Rana is an industrially experienced research scientist with a decade of experience in research and development within the pharmaceutical industry. She has a PhD degree in Biotechnology from the University of Natural Resources and Applied Life Sciences, Vienna (Austria) and holds an MBA in Entrepreneurship and Innovation from the Vienna University of Economics and Business (Austria).

Financials. InvolveMe will perform in 2017 its first conference-prototype with thrombotic thrombocytopenic purpura (TTP) as a rare disease because the founder researched a novel therapeutic solution for this disease during her PhD. The prototype will be performed in collaboration with the resources of the Ludwig Boltzmann institute, Vienna (Austria) within the frame of a one year workshop related to applying open innovation methods in science. InvolveMe will enter the market in Q1 2018 and will target in this year the 5 rare diseases receiving the largest number of clinical trials by various organizations. A net loss is expected for the first year and a breakeven will be reached in 2019. InvolveMe needs a financing of €350,000 to initiate its business. An initial funding will be made by the founders or through a grant from the EU's research and innovation framework program "Horizon 2020 projects". At the end of the third year, a gross margin of around 56% will be reached and stabilized.

		2018	2019	2020
	Conference broadcasting orders	18	66	106
	Number of Attendees from pharmaceutical companies	18	85	150
Sold services	Number of Attendees from Academia and Medical centers	45	133	198
	Revenues from Broadcasting	€ 268.500,00	€ 1.197.000,00	€ 2.010.000,00
	Revenues from Attendance by Pharma	€ 22.375,00	€ 66.500,00	€ 125.625,00
Revenues	Revenues from Attendance by Academia	€ 4.475,00	€ 13.300,00	€ 25.125,00
Sales Revenues Total		€ 295.350,00	€ 1.276.800,00	€ 2.160.750,00
Profit before Interest and Tax		-€ 136.630,00	€ 715.916,00	€ 1.337.626,00
Investment needed		€ 350.000,00	€ -	€ -
Employees		4	6	9

3.2 Business description

3.2.1 Mission Statement

“InvolveMe” is dedicated to close gaps in translational research by transiting patients with rare conditions from drug consumers to participants in research.

We democratize medical and pharmaceutical science research related to rare diseases to give patients whith rare conditions the opportunities to learn about innovations regarding their current diseases.

We help researchers to formulate research questions that coincide with projected patients´ concerns.

InvolveMe does not believe in biomedical innovation but in scientists, physicians and patients.

3.2.2 InvolveMe solution

“InvolveMe” is a for-profit organization that connects interactively patients with rare diseases to researchers researching the same disease at a global level through conferences addressing the latest innovations in medical and pharmaceutical sciences. InvolveMe employs crowdsourcing platforms and conferences to achieve its business goals (Figures 5 and 6).

3.2.2.1 Crowdsourcing of non- scientific abstracts

Step 1. InvolveMe invites researchers and physicians from Academia and pharmaceutical companies researching a specific rare disease to submit a non-scientific abstract in a jargon free manner for consideration by the patients affected by

this rare disease. Non-scientific abstracts refer to writing of abstracts that are not aimed to prove the validity of the research done but to communicate it to patients. InvolveMe provides researchers and physicians with instructions on how to communicate non-scientific results for non researchers in an easy manner (Figure 5).

Step 2. Through the crowdsourcing platform of InvolveMe, the concerned patients are invited to vote and to select non-scientific abstracts they would like to hear in an oral presentation or read through a poster. The best scoring abstracts according to the patient´s vote will be selected for oral presentation. Researchers and physicians receive confirmation of acceptance via E-mail for oral presentation or poster presentation based on the results of the patients´ votes (Figure 5).

Step3. Researchers and physicians selected will present their latest biomedical innovations in simple words in English for a duration of 10-15 min to an audience of stakeholders including but not limited to patients affected by the rare disease researched, physicians treating patients affected by the rare disease discussed, researchers from pharmaceutical companies and researchers from academia. The chairs of oral presentations would be a patients representative who likes to take that role together with a physician and a researcher selected by InvolveMe according to their knowledge in the topic presented. InvolveMe uses live video streaming to increase the reach of the conference to patients who cannot attend it and to customers mainly from the private sector (Figure 5).

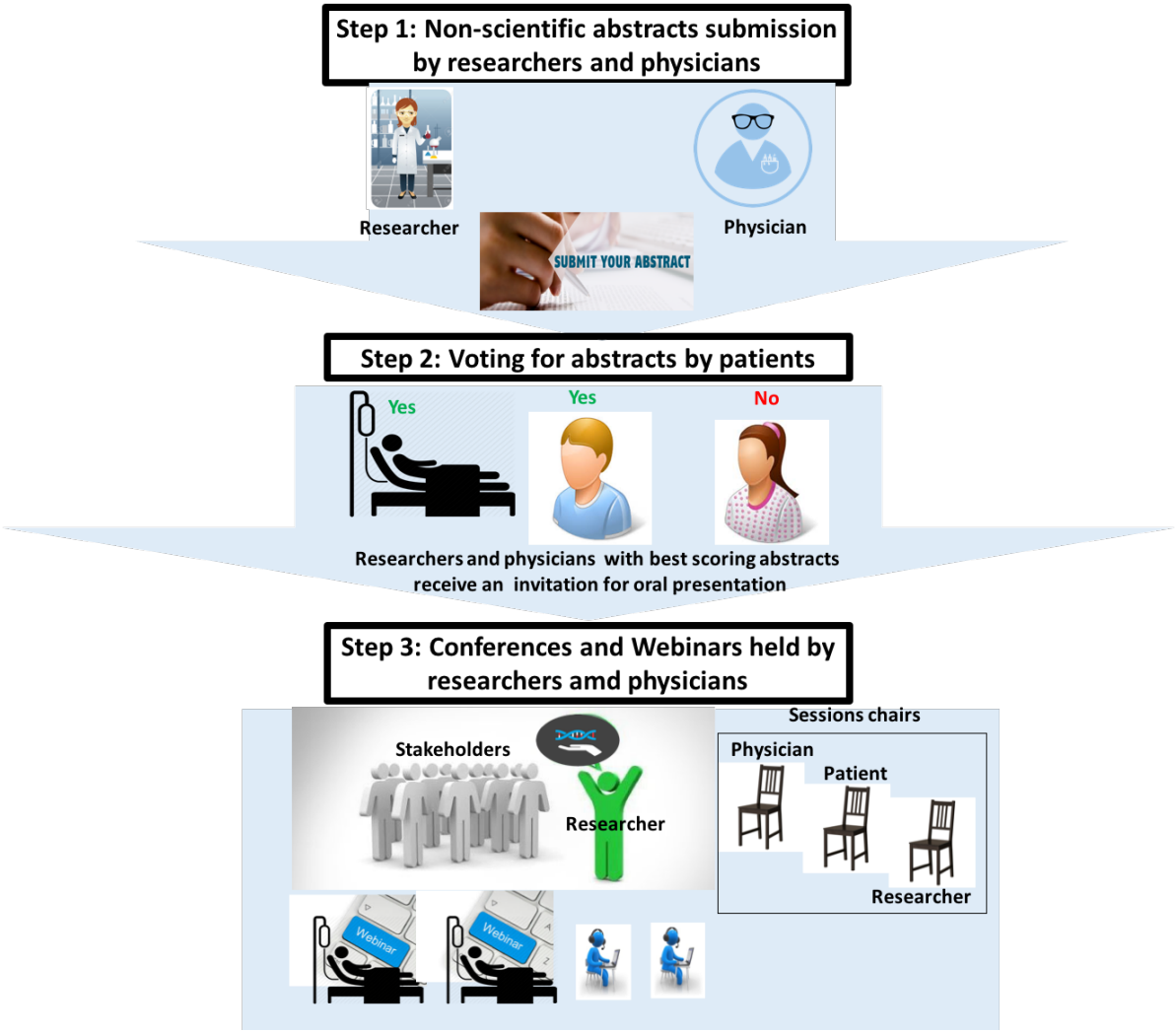


Figure 5. Crowdsourcing of non-scientific abstracts -Steps

3.2.2.2 Crowdsourcing of patients´ questions

Step 1. InvolveMe invites patients affected by a specific rare disease to submit anonymously a question that researchers need to answer regarding their disease. InvolveMe checks all submitted questions then make them available for the patient´s community (Figure 6).

Step 2. Patients are invited to vote on the questions they deem most relevant.

Step 3. Based on the patients´ votes, InvolveMe invites researchers and physicians to formulate and submit answers through InvolveMe platform to the 30 questions

deemed relevant by the patients´ community. Questions can be formulated by one individual or by a group of researchers and/or physicians (Figure 6).

Step 4. After the closing date of answers submission, and for each of the 30 questions, InvolveMe makes all answers anonymously available to the researchers´ community for voting. InvolveMe invites researchers and physicians to rate the answers submitted in an anonymous manner by their peers using a score from 1 (questionable answer) to 5 (valid answer). The three researchers whose answers score the best to each question are invited by InvolveMe to communicate their answers orally to the patients (Figure 6).

Step 5. Selected researchers and physicians are invited to answer the respective 30 patients´ questions in English for a duration of 10min per question to an audience of stakeholders including but not limited to patients affected by the rare disease researched, physicians treating patients affected by the rare disease discussed, researchers from pharmaceutical companies and researchers from academia. InvolveMe members voice the unanswered questions to the audience driving researchers to formulate new research questions to respond to patients´ unmet needs. Upon request, InvolveMe sells to regulatory agencies, policy makers and to funding agencies documented patients´ questions. Finally, researchers who participate to InvolveMe conferences receive a certificate of patient-centered scientist (Figure 6).

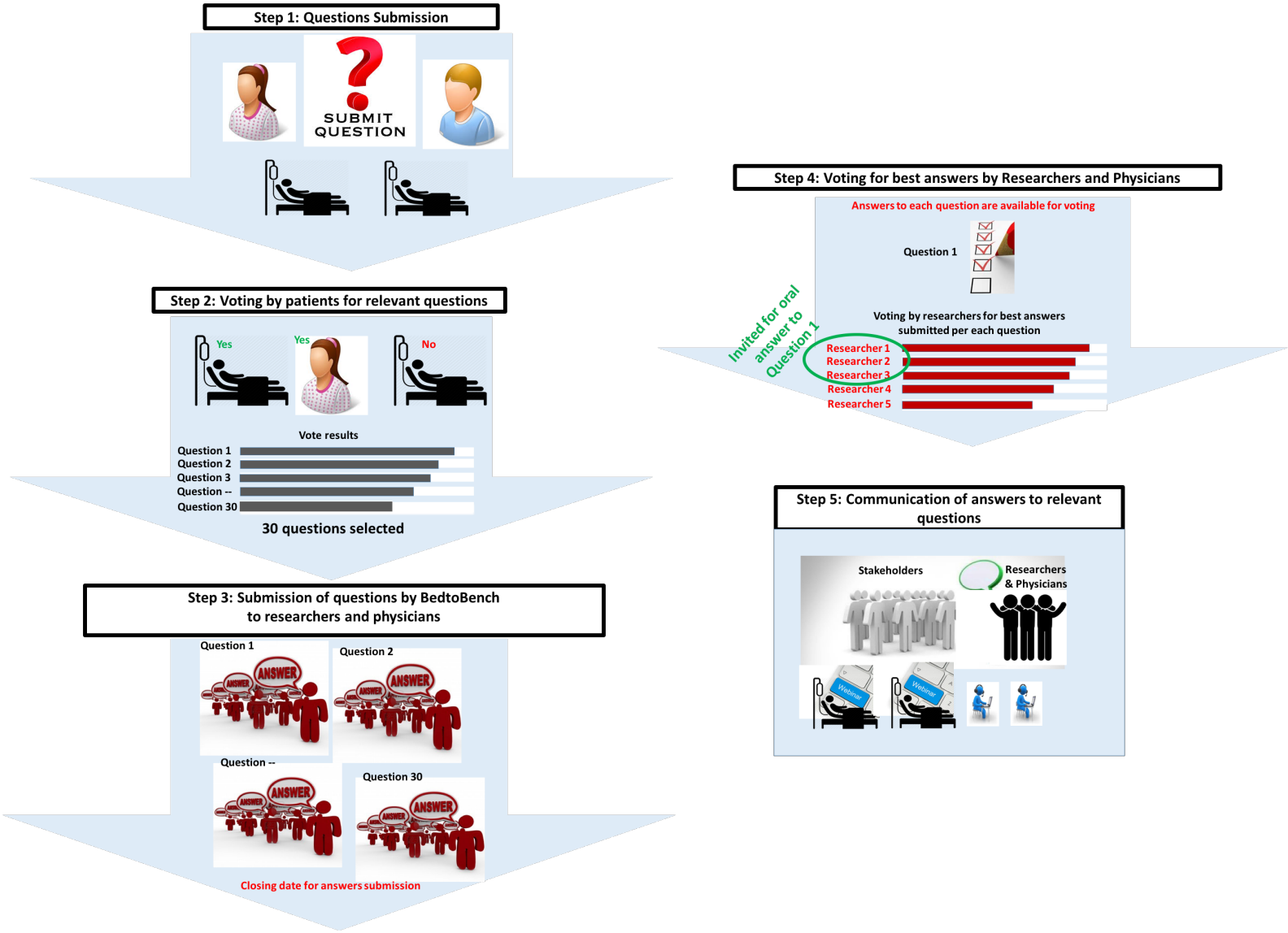


Figure 6. Crowdsourcing of patients´ questions-Steps

3.2.3 Services of InvolveMe

InvolveMe offers services to three major stakeholders which are i) the pharmaceutical industry ii) patients and iii) researchers and physicians from academia and medical centers.

3.2.3.1 Service for the pharmaceutical industry

InvolveMe offers the following services to the pharmaceutical industry:

- Direct connection of R&D team members researching rare diseases to patients´ communities with the same rare condition through conferences where patients´ concerns and innovations are shared and matched.
- Access to an exhaustive list of unanswered questions raised by patients reflecting end-users´ concerns.
- Ability to invite patients for clinical trials and to make patients survey.
- Cheap and compliant tool of patients-engagement while remaining business focused.
- Assistance by science journalists to write and communicate jargon free innovations stories to patients.

3.2.3.2 Service for patients with rare diseases and their supporters

InvolveMe offers patients affected with a specific rare disease, their family members and members of patients´ support organization the following services for free:

- Learn at a global level about the research activities of scientists regarding their disease.
- Raise unanswered questions to researchers.
- Transit smoothly from the consumer to the participant role in designing solutions for their own diseases.
- Possibility to join InvolveMe conferences either live or virtually.

3.2.3.3 Service for researchers and or physicians from academia and medical centers.

InvolveMe offers researchers from the public sector the following services:

- Access to a list of questions raised by the patients to formulate new research questions
- Ability for researchers to learn from patients about their journey with a rare condition.

3.2.4 Business Model

3.2.4.1 Value proposition

InvolveMe value propositions to patients with rare diseases:

- Learn in a jargon free manner about the latest innovations in biomedical and pharmaceutical sciences regarding their rare disease.
- Transit from drugs consumers to participants in research.
- Raise patients´ voice from bed directly to bench.

InvolveMe value propositions to the pharmaceutical industry focused on rare diseases:

- Cheap and compliant patient-centric solution.
- Quick distribution of research related survey to patients.
- Easy solution for patients´ recruitment for clinical studies.

InvolveMe value propositions to researchers from Academia and Medical centers:

- Provision of new research questions focused on patients´ needs.

3.2.4.2 Customers segments

The most important customers of InvolveMe are:

- Patients with rare diseases.
- Researchers from the pharmaceutical industry.

- Academics and Medical researchers from the public sector.

3.2.4.3 Channels

The channels that InvolveMe uses to reach its customers are:

- Phone calls and direct e-mails addressing by contracted post docs and InvolveMe employees to heads of research institutes and R&D departments at biopharmaceutical companies researching a specific rare disease.
- Sales channels of related non-competing businesses e.g. suppliers of diagnostic kits and Lab products to researchers in the public and private sector
- Patients´ support organizations.
- Social Media.
- InvolveMe website.

3.2.4.4 Customer relationships

The relationship of InvolveMe with each of its customers is developed through co-creation. InvolveMe depends entirely on researchers and patients in the creation of its services. InvolveMe offers only tools including platforms and live or broadcasted conferences so that researchers and patients come together around a common interest to share their professional and experiential knowledge. In this way, not only does InvolveMe form a personal relationship with its customers, but these bonds are strengthened by the additional relationships customers form with one another.

3.2.4.5 Key activities

The key activities that allow InvolveMe to generate revenue and in turn continue to improve the value it offers to its customers are:

- Platforms development ensuring that InvolveMe platform remains scalable and usable as the consumer base grows rapidly.
- Building strong relationships with patients´ support organization.
- Building strong relationships with researchers from the public and private sector to ensure that the platform is promoted to new users.

- Enabling communication of science in a jargon free manner to the widely scattered patients with rare diseases.

3.2.4.6 Key resources

The main input that InvolveMe will use to create its value proposition are:

- Well developed intelligence about rare diseases and orphan drug developers through contracted specialized postDocs per rare disease.
- Scalable crowdsourcing platforms.
- Global communities of researchers per rare disease as suppliers of non-scientific abstracts to InvolveMe.
- Global communities of patients and caregivers per rare disease as suppliers of research questions to InvolveMe.
- Human resources: Platform manager, Researchers and patients liaison, Science journalist and project manager.
- Grants.

3.2.4.7 Key Partners

The following partners are critical to the success of InvolveMe:

- Patients advocacy organizations for rare diseases e.g. NORD and EURORDIS.
- Suppliers of diagnostic kits and Lab products as a marketing channel for InvolveMe services.
- Research institutes for rare diseases e.g. Ludwig Boltzmann institute for undiagnosed rare diseases in Austria.
- Conferences and broadcasting organizers.
- Funding agencies for rare diseases.
- Pharmaceutical companies as sponsors.
- Science journalists.

3.2.4.8 Cost structure

InvolveMe me will focus completely on the value it will provide to the customers. The main costs are:

- Platforms development for abstracts/ questions submission and voting.
- Transaction platform to pay the registration fees
- Monthly salaries to employees.
- Overhead costs (PostDocs contracting, legal counseling, rent for office space, accounting and payroll fees, advertising expenses, telephone and electricity bills, travel expenditures).
- Events related expenses (Broadcasting and Conference venues).

3.2.4.9 Revenue streams

InvolveMe will generate 90% of its revenue through broadcasting its jargon free conferences to sponsors of clinical trials, the remaining 10% will be from registration fees paid by any person who registers on its platforms except patients, their family members and members of patients support organizations. Once the business is successful, InvolveMe will have new sources of revenues from advertisement, access to recorded conferences and documented patients´ questions to regulators and funding agencies.

3.3 Description of industry

3.3.1 Industry Analysis

3.3.1.1 General trends regarding patients´ involvement in R&D

A number of public and private initiatives have begun to involve patients in R&D activities and to increase the health literacy. These initiatives are discussed below:

3.3.1.1.1 Patients training in research methods

In 2014, a European innovative medicines initiative initiated a project named “The European Patients’ Academy (EUPATI). This project focuses mainly on education and training of patients to increase their capability to understand and contribute to medicines research and development in general without any focus on disease-specific issues or therapies. The project is led by the European patients’ forum, with partners from patient organizations, universities and not-for-profit organizations, along with a number of European pharmaceutical companies.

Another joint initiative between Alberta Health Services (AHS) and the Institute for Public Health at the University of Calgary was established in 2013. This initiative aims to train patients in formal research methods. Once they graduate, these volunteers become patient-engagement researchers and join AHS’ strategic clinical networks in order to determine how the health system can deliver high-quality, patient-centered care[(15)].

These trends clearly indicate that the business idea of InvolveMe goes in the same direction as that of the joint initiatives described above.

3.3.1.1.2 Co-development of clinical trial protocols with patients

Another example is the one of “Transparency Life Science” whose mission is to invite patients to develop their own clinical trial protocols. Transparency Life Science is a clinical-stage drug development company based on open innovation. It develops the clinical protocol for drugs with crowdsourced input from researchers, physicians and patients.

3.3.1.1.3 Patients and Citizen involvement in suggesting new research topics

In Austria, the Ludwig Boltzmann Gesellschaft recently geared research strongly towards the needs of people. Last April 2015, this research institute initiated a

crowdsourcing project named “Tell us” for which patients, families, health care professionals, physicians, and therapists were invited to suggest new research topics[16]. The contributions were analyzed independently of all economic interests. The results have been communicated to policy makers and for the first time the scientific community served in Europe as a basis for future research projects. Interestingly, the Ludwig Boltzmann Institute founded this year a research institute for rare and undiagnosed diseases. Hence, this institute may be a partner for InvolveMe especially after having run the “Tell us” crowdsourcing project discussed above.

Similarly, in the United States, scientists asked the public to join them in their quest to mine the Earth’s soil for compounds that could be turned into vital new drugs[17].

Together, the “Tell us” project and the “earth soil” project indicate that the trend in R&D today is to engage as early as possible with the public.

3.3.1.1.4 Leveraging patients´ data on social network platforms

Today patients want to engage in a process that extends beyond self-discovery to manage their disease conditions and associated symptoms. Hence, several social networks or health sharing platforms like Facebook, PatientsLikeMe.com and Smart Patients.com empower patients through self-tracking and/or conversations. One of the best example here is PatientsLikeMe that is a for profit company co-founded in 2004 by three MIT engineers who built a health data-sharing platform that transformed the way patients manage their own conditions. Patients share there their real-world health experiences in order to help themselves, other patients like them and patients´ advocacy organizations that focus on their conditions. Interestingly, PatientsLikeMe aligned patients and industry interests through data-sharing partnerships. Hence, industry partners who can afford the costs of such partnerships, use the patients´ health data to improve products, services and care for patients. The techniques used for these insights are quantitative analysis and natural language processing. Compared to patientslikeme, InvolveMe would offer less virtual and friendlier connections between researchers and patients.

3.3.1.2 General trends regarding the attention on rare diseases

Let´s first define the words “Rare disease and orphan drugs”.

In EU countries, any disease affecting fewer than 5 people in 10 000 is considered rare. That number may seem small, but it translates into approximately 246 000 people for one rare disease throughout the EU’s 28 member countries. Most patients suffer from even rarer diseases affecting 1 person in 100 000 or more. It is estimated that today in the EU, 5000-8000 distinct rare diseases affect 6-8% of the population – between 27 and 36 million people[18].

“Orphan drug” is a pharmaceutical agent that is developed specifically to treat a rare medical condition. Today, there is a big attention on rare diseases from the pharmaceutical industry, policy makers and various patients´ support groups have been created.

3.3.1.2.1 Focus of pharmaceutical companies on rare diseases

For decades, pharmaceutical industries did not invest in rare-disease treatments because they focused on mass-market drugs for common problems. Then, starting a decade ago, patents on these medicines began to expire, and got replaced by cheap generic drugs. Therefore, the pharmaceutical industry began to pour billions of dollars into developing innovative therapies for rare diseases. Furthermore, several other factors converged: 1) improved understanding of rare disease biology owing to the mapping of the human genome 2) drugs for rare disease are tested on much smaller groups leading to faster drug approval 3) government financial support for orphan drug development and finally 4) the influential patient-advocacy groups that raise money for research and build registries of patients to allow recruiting patients for drug studies.

3.3.1.2.2 Current policy makers´ actions related to rare diseases

European policy makers. A number of initiatives by the European commission have begun to further improve the access to prevention, diagnosis and treatment for patients suffering from a rare disease. A brief list of these initiatives is shown below:

- Creation of ORPHANET database which lists the descriptions of almost 6000 rare diseases.
- Establishment of European Reference Networks (ERNs) to facilitate cooperation between member states in the development of diagnosis and treatment for rare or low prevalence complex diseases or conditions.
- Commitment to funding research in rare diseases. The Horizon 2020 work program 2015 for “Health, demographic change and wellbeing” includes a budget of € 62 million euros for developing new therapies for rare diseases.
- Creation of the International Rare Diseases Research Consortium (IRDiRC) as the biggest collective rare diseases research effort worldwide together with European and international partners. IRDiRC key objective is to deliver, by 2020, 200 new therapies for rare diseases and the means to diagnose most of them.
- Creation of a European platform on Rare Diseases Registration. The goal of the registries is to document natural history data to help medical researchers better understand how diseases develop and progress over time.
- Help organizations that support patients. The European Commission provides a grant to the European Organization for Rare Diseases (EURORDIS), a non-governmental patient- driven alliance of patient organizations.

American policy makers. Similarly, the FDA's Office of Orphan Products Development (OOPD) set a grant program to support and encourage the clinical development of products for use in rare diseases. Another program was set by OOPD to fund targeted natural history studies for rare diseases. The goal of the orphan products natural history grants program is to support studies that advance rare disease medical product development through characterization of the natural history of rare diseases/conditions, identification of genotypic and phenotypic subpopulations, and

development and/or validation of clinical outcome measures, biomarkers and/or companion diagnostics[19].

3.3.1.2.3 Creation of patients´ support groups for rare diseases

Two non-governmental leading patients support groups represent the voice of European and American patients with rare diseases.

In Europe, EURORDIS is a non-governmental community of patient organizations and people living with rare diseases. It represents 614 rare disease patient organizations in 58 countries, and is the voice of 30 million people affected by rare diseases throughout Europe.

In the US, the National Organization for Rare Disorders (NORD) provides advocacy, education, patient services and research to address the needs of Americans with rare diseases. NORD was instrument in the Orphan Drug Act of 1983, which created government financial incentives through US law for the development of treatments for rare diseases.

Looking at the trends regarding the attention on rare diseases, InvolveMe has to carefully consider how to position its new services in the mind of policy makers, patients support groups and researchers from the public and private sectors.

3.3.2 Industry forecast

No data were available to estimate the total number of researchers from the public and private sector researching rare diseases. However, a European register of all designated orphan medicinal products and their sponsors is available at the European commission website[20]. The data from this website were exported to an Excel sheet to estimate the number of institutions or pharmaceutical companies (named also “sponsors”) who perform a clinical trial for each registered orphan drug in development. The total number of sponsors between 2000 and 2016 was found to be 671. In other terms, InvolveMe would have to connect with maximum 671 sponsors.

Most of these sponsors are from the private sector and perform clinical studies for more than one orphan designated drug.

Based on market research data, the industry of orphan drugs is expected to grow at a compound annual growth rate (CAGR) of around 10.5% from 2014-2020. The global orphan drug market was around $97 Billion in 2014, and is anticipated to grow to around $176 Billion by 2020[21]. Orphan drugs are set to account for 19.1% of global prescription sales (Figure 7) in 2020, excluding generics, up from 6.3% in 2000.

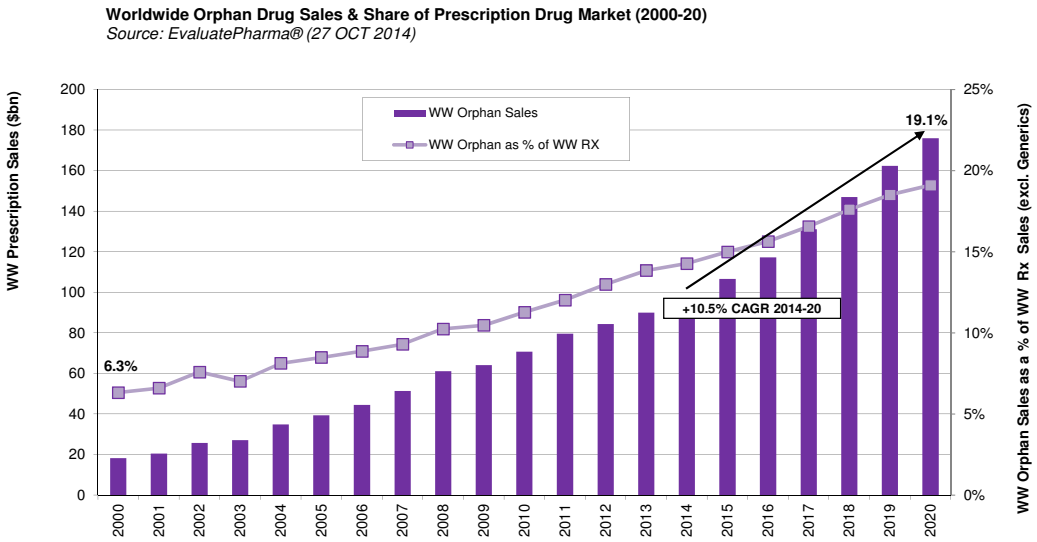


Figure 7. Worldwide Orphan drug sales & share of prescription Drug Market (2000-2020)

The number of orphan drugs under a clinical process is also increasing. In 2014, the number of US designations medicines under R&D as orphan medicines increased 38% in 2013 to 260, the highest number of designations seen so far, reversing the decline seen in 2011. European orphan designations declined 17% to 124 (Figure 8).

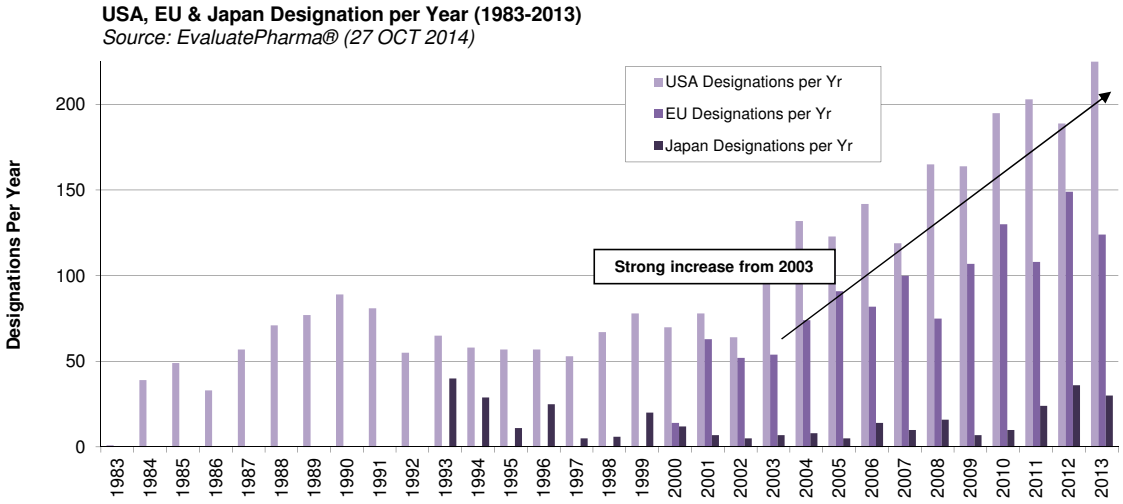


Figure 8. USA, EU, Japan Designation of orphan drug per Year (1983-2013)

3.3.3 Analysis of competitors

No one has initiated such an initiative to give patients with rare disease the opportunities to learn about innovations in medical and pharmaceutical science. Furthermore, there is no service provider dedicated to inject an intangible and sticky experiential knowledge of patients in biomedical research. To my knowledge, the only group that is leveraging the patients´ knowledge is "Patient Innovation"[(22)], a nonprofit, international free venue for patients and caregivers of any disease to share their own invented solutions. However, this group does not support translational research as it is only focused on products that patients invent to solve their own problems. With that in mind, a list of groups that could be considered as competitors is provided.

3.3.3.1 Patients´ support organization

Currently, patients´ support organizations offer symposium connecting mainly doctors across the world and patients and organize patient education day. For instance, the Answering thrombotic thrombocytopenic purpura (TTP) foundation, a patients´ support organization with whom I was previously in contact, updates patients with TTP about developments in TTP treatment and TTP research at a global level. However, in its education offer, the physician community involves very few researchers from the public sector and no pharmaceutical companies are present during such events.

Hence, compared to the education days offered by patients´ support organizations, InvolvMe considers that the knowledge shared during its conferences is more exhaustive, holistic and original.

3.3.3.2 Social platform- Patientslikeme

PatientsLikeMe offers expensive data-sharing partnerships with life science organizations who can afford the costs of such partnerships. It provides a virtual discourse between researchers and patients whereas InvolveMe brings the patients´ voice live to researchers. Next, while leveraging the patients´ data on PatientsLikeMe platforms using algorithms and language processing softwares, pharmaceutical companies are more business focused than patients centric. However, by using involveMe services, companies can be in the sweet spot of being patients-centric and business focused. The business focus would stem in this situation from being able to i) call openly for clinical studies ii) listen to what the competitors are sharing with the same patients´ communities and iii) attract investors through the jargon free innovations stories communicated. Last but not least, when using InvolveMe services private organizations cannot be accused by doing lobbying operations because conferences are organized by InvolveMe as a third party.

3.3.3.3 Physicians

Because knowledge is power, it is possible that physicians dislike the activities of InvolveMe as they will not anymore be the main source of information regarding a specific field of research in rare diseases. However, InvolveMe considers its activities as a great tool for physicians but also for nurses to learn at a glance about innovations that are occurring regarding a rare disease.

3.3.3.4 Medical science liaison staff

Medical science liaison (MSL) is a specific employee role within the bio/pharmaceutical industry. MSLs have academic credentials generally consisting of a doctorate degree in life sciences. They concentrate on a specific therapeutic area

(i.g. Oncology, Cardiology, etc.) and are vital in the success of a pharmaceutical company as they work throughout a product's lifecycle. Their primary purpose is to establish and maintain peer to peer relationships with leading physicians, referred to as Key Opinion Leaders (KOLs) at major academic institutions and clinics. With the business idea of InvolveMe, researchers who present their research activities to an audience of patients but also physicians and KOLs can affect and dilute the role of MSLs.

3.3.3.5 European Conference on Rare Diseases & Orphan Products

The European Conference on Rare Diseases & Orphan Products (ECRD) can be considered as a competitor in Europe since it is the only forum across all rare diseases and across all European countries, that brings together through scientific conferences all stakeholders: patients' representatives, academics, health care professionals, industry, payers, regulators and policy makers. InvolveMe adds a new nuance to ECRD scientific conferences: InvolveMe business model is focused on exchanging non-scientific stories between researchers and patients whereas ECRD brings all stakeholders together around scientific presentations. Additionally, patients´ organizations members pay a fee of 170€/ member for attending ECRD whereas InvolveMe offers its conference to the customer for free.

3.3.3.6 Conference on Clinical Research for Rare Diseases

The Conference on Clinical Research for Rare Diseases (CCRRD) is an American initiative that is analogous to the ECRD discussed above. The CCRRD initiative is made up of 22 research groups and a data management and coordinating center that are working together to improve availability of rare disease information, treatment, clinical studies, and general awareness for both patients and the medical community in the United States. Again, here CCRRD does not bring its stakeholders together around non-scientific innovations as InvolveMe does.

3.3.4 SWOT analysis

Strengths.

- First service provider of jargon free biomedical innovation stories to the public.
- First provider of patients´ unanswered questions to formulate new research questions.
- Strong perceived value by patients.

Weaknesses.

- High bargaining power of researchers and patients as suppliers.
- Low barrier to entry.
- Scientists, physicians don´t want to train jargon free presentations.
- High variable costs.
- Dealing with patients´ data and continuous costs related to legal counseling.
- Voting of abstracts by patients may discourage competing sponsors to participate due to the fear of being rejected by the end-users.
- Too much open project for a closed ecosystem.

Opportunities.

- Potential support by policy makers as the initiative refers to open innovation in science and to rare diseases.
- Becoming publisher of articles related to non-scientific biomedical innovations.
- Target all rare diseases and become the preferred third party provider of patient-centric solutions for the pharma industry.
- Strong perceived value for investors and banking sector.
- Initiate GoPharma initiative to organize visits of patients to pharmaceutical industries.
- Initiate BedtoBench initiative where patients test with researchers their own biological samples or co-develop diagnostic tests with manufacturers of diagnostic kits.
- Initiate ChildrenLabs initiative to familiarize children with rare condition to research in the future their own disease.

Threats.

- Patients support organization could feel substituted.
- Difficult market entry due to weak network with key leaders in rare disease research.
- Presence of other social platforms (e.g. patientslikeMe) where patients and researchers communicate e.g. patientslikeme.
- Opposition by physicians as they may feel substituted.
- Promising patients with solutions that may not come to the market

To transform threats into opportunities, InvolveMe must partner with patients´ support organizations, get endorsed by policy makers and set an international advisory board.

3.4 Technology plan

Various platforms and technologies are required to service researchers and patients effectively. The technology plan includes 1) crowdsourcing platforms either for informing patients about research activities or for obtaining researchers´ answers regarding patient´s relevant questions 2) broadcasting technologies to increase the reach of the conference to patients who cannot attend and to customers from the private sector and finally 3) a platform allowing transactions revenues.

3.4.1 Crowdsourcing platforms

The development of the crowdsourcing platforms will be outsourced. Upon registration patients are requested to add their personal data. Participants indicate whether they are patients, family members of the patient, members of patients support, researchers from the pharmaceutical industry or academia, physicians, policy makers or investors.

3.4.2 Broadcasting technologies

This technology is outsourced to a third party that is selected according to the location of the conference.

3.4.3 Transactions platform

People register on InvolveMe platform through the internet. Using PayPal or credit cards, every registered person, except patients, caregivers or members of patients´ advocacy organization, pay a fee for registering at InvolveMe conferences.

3.5 Marketing plan

3.5.1 Target Market

The targeting strategy of InvolveMe is rare disease based and not orphan-drug or industry based. In other words, InvolveMe will not target industries or research centers who develop orphan drugs like Novartis, Shire, Pfizer etc. Instead, InvolveMe will target rare diseases that attract the largest number of sponsors of clinical trials. Based on the European register of all designated orphan medicinal products under clinical development[20], the list of treatments in development per rare disease were grouped per descending number of sponsors, a sponsor being defined as an individual, a company, an institution or an organization which takes responsibility for the initiation, management and/or financing of a clinical trial. The first 15 rare diseases with the largest number of sponsors are shown in Table 3.

Table 3. Rare diseases to be targeted by InvolveMe (2018-2020)

Designated Orphan indication	Number of sponsors*
Treatment of acute myeloid leukaemia	44
Treatment of glioma	36
Treatment of cystic fibrosis	36
Treatment of pancreatic cancer	31
Treatment of ovarian cancer	28
Treatment of chronic lymphocytic leukaemia	20
Treatment of hepatocellular carcinoma	18
Treatment of acute lymphoblastic leukaemia	17
Treatment of Duchenne muscular dystrophy	17
Treatment of amyotrophic lateral sclerosis	15
Treatment of retinitis pigmentosa	15
Treatment of mucopolysaccharidosis	14
Treatment of cutaneous T-cell lymphoma	14
Treatment of systemic sclerosis	14
Treatment of haemophilia	12

* Refer to Appendix 1 for the identity of sponsors per rare disease.

The strategy of InvolveMe is to target 5 rare diseases per year (Table 4). InvolveMe considers that the advantage of such targeting strategy is that as soon as one sponsor participates to InvolveMe crowdsourcing sessions, competitors will join afterwards to remain updated with the activities of the other players.

Table 4. Targeting strategy, InvolveMe (2018-2020).

			Designated orphan indication	Total Sponsors
			Treatment of Thrombotic thrombocytopenic purpura*	4
			Treatment of acute myeloid leukaemia	44
			Treatment of glioma	36
			Treatment of cystic fibrosis	36
			Treatment of pancreatic cancer	31
		2018	Treatment of ovarian cancer	28
			Treatment of chronic lymphocytic leukaemia	20
			Treatment of hepatocellular carcinoma	18
			Treatment of acute lymphoblastic leukaemia	17
			Treatment of Duchenne muscular dystrophy	17
	2019		Treatment of amyotrophic lateral sclerosis	15
			Treatment of retinitis pigmentosa	15
			Treatment of mucopolysaccharidosis	14
			Treatment of cutaneous T-cell lymphoma	14
			Treatment of systemic sclerosis	14
2020			Treatment of haemophilia	12

* Thrombotic thrombocytopenic purpura refers to the first prototype of InvolveMe that is foreseen to be targeted in 2017 and carried over in the subsequent years - see next section.

The first prototype of InvolveMe. Due to my professional experience in thrombotic microangiopathies gained during my PhD, the first prototype of InvolveMe will be Thrombotic thrombocytopenic purpura (TTP). TTP conferences will be performed in 2017 and maintained when InvolveMe starts its business in 2018. It is very likely that the first prototype of InvolveMe will be performed in collaboration with the resources of the Ludwig Boltzmann institute, Vienna (Austria) within the frame of a one year workshop related to applying open innovation methods in science[(23)]. InvolveMe customers´ list for TTP is shown in Table 5.

PostDocs who researched Acute myeloid leukemia, Glioma, Cystic fibrosis, pancreatic cancer and ovarian cancer (referring to the 5 Rare diseases to be targeted in 2018) will be contracted by InvolveMe to create customers´ list for each rare disease.

Table 5. Key stakeholders of "InvolveMe", Prototype-TTP

Pharma Companies (n=4)	**Focus**	**Location**
Ablynx	Develops a drug called "Caplacizumab"	Belgium
Omeros	Develops a drug called OMS721	Seattle
Shire	Develops a drug called Recombinant ADAMTS13	Austria
Glenmark Pharmaceuticals	Develops a drug called GBR 600 (anti-platelet monoclonal antibody)	UK
Academia (18 independent centers)	**Focus**	**Location**
University Clinic of Hematology and Central Hematology Laboratory Inselspital, University Hospital and the University of Bern	Hematology	Switzerland
University College London Hospital, London, UK	Hematology	UK
Katholieke Universiteit Leuven Campus Kortrijk	Thrombosis Research and Molecular/Vascular Biology	Belgium
Angelo Bianchi Bonomi Hemophilia and Thrombosis Centre, University of Milan	Hemophilia and Thrombosis	Italy
Department of Hemostasis, Aesculabor, Hamburg	Hemostasis	Germany
Center for Thrombosis and Hemostasis (CTH), University Medical Center Mainz, Mainz, Germany	Thrombosis and Hemostasis	Germany
Sanquin Research (not-for-profit organization)	Blood transfusion medicine and immunology	Netherlands
Service d'Hématologie et de Thérapie Cellulaire, Hôpital Saint-Antoine, AP-HP; UPMC Univ 06, Paris	Hematology	France
AKH Wien	Hematology	Austria
Department of Haematology, St Olavs Hospital Trondheim University Hospital, Trondheim, Norway	Hematology	Norway
University of Oklahoma Health Sciences Center, Oklahoma City, USA	Biostatistics and Epidemiology	USA
The Children's Hospital Philadelphia and The University of Pennsylvania, Philadelphia, PA	Pathology and Laboratory Medicine	USA
Howard Hughes Medical Institute, Washington University School of Medicine, St. Louis, MO	Medicine, Biochemistry and Molecular Biophysics	USA
Albert Einstein College of Medicine and Montefiore Medical Center, Bronx, New York	Hematology	USA
Department of Internal Medicine Fellow, Medical Oncology & Hematology, The Ohio State University	Internal medicine, Medical Oncology & Hematology	USA
Nara Medical University, Nara	Blood Transfusion Medicine	Japan
Department of Laboratory Medicine, Keio University School of Medicine, Tokyo	Laboratory Medicine	Japan
National Cerebral and Cardiovascular Center, Suita, Osaka	Molecular Pathogenesis	Japan
Patients support group	**Focus**	**Location**
Answering T.T.P. Foundation (a NORD member organization)	Raise funds for TTP research, Patient support & Education, Medical Research Collaboration	Canada, UK
Social Media platform	**Focus**	**Location**
TTP supporters group with 1500 members on Facebook	Help connect and support the geographically dispersed patient and supporter population	Social media channels

Table 5 shows that for the treatment of TTP, the disease despite being rare attract 4 sponsors and 18 research centers and hospitals performing research activities related

to TTP. Hence, in the financials section, I will assume that in general one rare disease is researched at least by 15 research centers from the public sector. The exact current centers will be identified with the help of the contracted PostDocs and the list of the European research centers per rare disease available on the webpage of Orphanet[24].

3.5.2 Marketing mix variables

3.5.2.1 Services

InvolveMe services are aimed to connect directly patients with rare diseases to researchers researching the same disease. The bundle of features and benefits to the stakeholders that makes the service of InvolveMe distinctive from other existing competitors was described earlier under the chapter “Business description/Services of InvolveMe”- section 3.2.3.

3.5.2.2 Price

InvolveMe will offer services to three main stakeholders who are the patients, pharmaceutical industry and academics including physicians. Prior to setting the price, the yearly total costs for InvolveMe were estimated (Table 6). Table 6 shows that the costs of delivering 16 conferences covering 16 various rare diseases during 3 years are €1.959.980 (€431.980+€560.880+€967.120) say €122.498 per rare disease (€1.959.980 /16).

Table 6. Operational expenses for 16 rare diseases (2018-2019), (€)

Operational Expenses	2018	2019	2030
Salaries[(1)]	200.000	310.000	475.500
Legal counseling[(2)]	20.000	30000	45000
Contracted Post-Docs [(3)]	50.000	50000	50000
Fringe benefits[(4)]	64.000	99.200	152.160
Rent office, Utilities[(5)]	14.400	18000	21600
Advertising[(6)]	16.440	2.160	3.240
Telephone and Internet[(7)]	2.400	3600	5400
Depreciation of platforms (Crowdsourcing, Transactions) [(8)]	5.000	5000	5000
Computers, Printer and office accessories [(9)]	8.100	5940	8700
Outsource accounting and payroll[(10)]	3.100	3100	6200
Unexpected expenses	10.000	15000	22500
Video Life streaming[(11)]	2.500	5000	7500
Conference permise and catering up to 30 people[(12)]	6.000	11000	160000
Ticket and hotel fees [(13)]	1.440	2880	4320
Legal setting, InvolveMe	25.000	-	-
3-month deposit for rent	3.600	-	-
TOTAL COSTS	431.980	560.880	967.120

(1) In 2018, four salaries for 4 employees (average €50.000 each) as Platform manager, Patient and researcher liaison (myself), Science journalist, Project manager. When the business scales up, two new employees join InvolveMe in 2019 then 3 other employees in 2020.
(2) Legal counseling, €20.000 in 2018. The sum increases by 50% in 2019 then by another 50% in 2020
(3) In 2018, one PostDoc per rare disease except for Thrombotic thrombocytopenic purpura (€10,000€/rare disease-→ for five diseases €50,000), PostDocs are only contracted when the disease is targeted for the first time.
(4) Rough estimation according to the Austrian law: 32%
(5) €12/m^2 rent + €3/m^2 for heat and electricity; estimated required surface in 2018 is 80m^2-→ rent/month is 80x15=€1200. This rent increases in 2019 and 2020 due to the increasing number of employees.
(6) Website setting 10000€, 5000€ for a hand-drawn explanation video on the platform to communicate InvolveMe mission, €30/month/employee membership to LinkedIn
(7) Unlimited cell phone - €50/month/employee
(8) The platforms of InvolveMe are expected to cost 50000€. The platforms are depreciated over 10 years leading to a depreciation amount of €5000 per year.
(9) Computer and printers are leased (€35/computer/employee/month, €35/month for leasing a printer, €1500 paid once per employee for office furniture).
(10) €2500/year for accounting and €50/month for payroll in 2018. This sum remains the same in 2019 and doubles in 2020 due to the increase of employees.
(11) The cost of video livestreaming was taken from the company "Livestream" and this is only a fee to pay per one month. Hence, to reduce its variable costs, InvolveMe will organize every 5 conferences in the same month. As 5 new diseases are targeted each year, the costs of broadcasting becomes €10.000 in 2018 and €15.000 in 2020.
(12) For each rare disease, there is only one conference for one day. Costs are €1000/day including catering. For 6 rare diseases, total costs €6000 in 2018, then €11000 for 11 rare diseases in 2019 and €16000 for 16 rare diseases in 2020.
(13) 1 person from InvolveMe to join the conferences per rare disease. (€600/ticket and €120 hotel costs per day for seven days. Costs are 600+120X7= €1440 per set of 5 rare diseases targeted in 2018.

The number of participants per year at InvolveMe including the broadcasting orders were anticipated based on a market penetration per year projected as follows:
- for the broadcasting orders: 10% of the total number of sponsors in 2018, 30% in 2019, 40% in 2020
- for the attendance from the private sector: 25% of the total number of sponsors in 2018, 50% in 2019, 75% in 2020.
- for the attendance from the public sector: 10% of the total number of research centers, 20% in 2019, 30% in 2020. Table 7 depicts a summary of the total anticipated number of participants and broadcasting orders.

Table 7. Anticipated number of participants and broadcasting orders (2018-2019)

	2018	2019	2020	Total
Broadcasting orders	18	80	134	**232**
Number of Attendees from pharmaceutical companies	45	133	251	**429**
Number of Attendees from Academia	18	53	101	**172**

According to Table 7, and between 2018 and 2020, each rare disease related conference can be estimated to be broadcasted to 15 private companies (Total number of broadcasted conferences in three years/16 rare diseases; 232/16=15). Similarly, between 2018 and 2020, the total number of attendees from the private and public sector is 601 (429 from the private sector and 172 from the public sector), say 38 attendees per rare disease (601/16 rare diseases= 38; 27 from the private sector and 11 from the public sector). Thus, to determine the cost per broadcasted conference and per attendee, two sales scenarios can be foreseen: i) 38X=€122.498 where X refers to the costs per one attendee or ii) 38X+15Y=€122.498 where X refers to the costs per one attendee and Y to the costs per one broadcasted conference. The first scenario 38X=€122.498 is impossible as it means that the approximate cost per attendee is €3224. When considering the role of the competition, and taking into account the cost of one of the best scientific conferences in the field of blood disorders for example (e.g. The International Society on Thrombosis and Hemostasis named ISTH), the cheapest attendance price regardless the professional sector of the participant is $900/person. Similarly, the costs of attending the European Conference

on Rare Diseases (ECRD) conference are 1200€ from the private sector and 310€ from the public one.

As InvolveMe conferences aims to get a high number of participation of all stakeholders to increase the co-created value of its services, the pricelist set by InvolveMe must not exceed €500/person, say €500 for the private sector and 250 for the attendees from the public sector, the latter price being selected intentionally low as the motivation of this sector to join InvolveMe conferences is low. Consequently, InvolveMe has to stream its revenues from broadcasting. Here, using the scenario 38X+14Y=€122.498 where X=€427/person instead of €500 [427= (500x27+11x250)/38 because among the 38 total participants 11 are from the public sector and 27 from the private sector], the cost of one broadcasted conference is 7590€. Adding a markup of 200% would set the final price of one broadcasted conference to €15.181, say €15.000.

The pricelist set by InvolveMe is depicted in Table 8. In view of its mission, InvolveMe will not charge patients and organizations that support them. The pricelist for attending the conference of InvolveMe is set for €250 for researchers from the public sector and €500 from the private sector with incentives for early registration.

Table 8. Registration fees at InvolveMe

	Crowdsourcing sessions and conferences		Broadcasting fees		
Category	Registration two months before the initiation of crowdsourcing sessions	Registration at the first day of crowdsourcing sessions	Registration two months before the conference date	Registration at the same conference date	Access to recorded conferences
Patients, Family members, Patients' Organizations	Free	Free	Free	Free	-
Academics and students* / Health care professionals from the public sector / policy makers / payers / regulators	250€	312.5€	5000€	5500€	-
Pharmaceutical industry, Suppliers of lab products, consultants, investors	500€	635€	15000€	16500€	-
Anybody except patients	-	-	-	-	30000€

* including medical or PhD Students / Trainees (Medical, Bachelor, Master or PhD) as well as Medical residents and post docs defined as any person holding a doctoral degree who is engaged in a temporary period of clinical training and/or mentored research and/or scholarly or practical training for the purpose of acquiring specific professional skills and who is not an independently practicing clinician.

3.5.2.3 Place

InvolveMe delivers its service on a continuous basis during a period of 6 months. Participants register through its webpage and use its crowdsourcing platforms immediately after registration until the date of the conference event. InvolveMe streams online its conferences to registered patients and to industries. Access to recorded conferences by interested stakeholders is available on demand after the event.

After the conference, registered participants receive full access to an exhaustive list of i) non-scientific abstracts presented ii) the list of patients´ questions that research can answer as well as those questions deemed relevant in the view of the patient´s community iii) the best scored researchers´ answers to the questions that could be answered and finally vi) the patients´ questions that remain orphan without answers.

3.5.2.4 Promotion

Prior to starting its business, it is essential that InvolveMe partners with suppliers of Lab materials and patients´ support organization to promote its services to patients and researchers.

3.5.2.4.1 Promotion through suppliers of diagnostics kits and lab products

Involve me will back-ride on the sales channels of suppliers of “diagnostic kits and Lab products” to researchers in the public and private sectors through their well-established relationships with customers. Hence, such partnerships must be established prior to starting the business. InvolveMe customers´ lists created by the contracted PostDocs will be shared with the sales representatives of these suppliers to communicate and promote InvolveMe platforms and conferences. To create a win-win situation, suppliers of diagnostics kits and lab products will receive in turn free advertisement services during the crowdsourcing sessions and the conferences of InvolveMe.

3.5.2.4.2 Promotion through patients´ support organization

InvolveMe will contact the respective patients´ support organizations per rare disease targeted. For example, to promote its first prototype, InvolveMe will contact the “Answering TTP foundation”.

To reach patients with other rare diseases, InvolveMe will mainly rely on EURORDIS and NORD considered to be the nodes of patients´ support organizations for rare

diseases in the European union and in the US. Hence, partnerships with EURORDIS and NORD must be established prior to starting the business.

3.5.2.4.3 Phone calls and e-mails addressing

To ensure high number of participation, InvolveMe will complement the marketing activities of the suppliers of lab materials and patients´ support organizations by phone calls and e-mails addressed by its employees.

For each newly targeted rare disease, InvoveMe will contract postDocs for a period of 2 to 3 months to establish a customer list similar to the list developed for TTP (Table 5). E-mails will be addressed to all heads of research institutes and R&D departments researching the rare diseases targeted. Potential attendees from research centers will be contacted by personal phone calls to get informed about the crowdsourcing sessions and conferences of InvolveMe.

Each employee of InvoleMe will have a membership to LinkedIn. Through LinkedIn InMails, InvolveMe employees will send personal Emails to registered researchers and head of patients´ relations and communications in the pharmaceutical companies. InvolveMe will also ask researchers to promote its conferences via their distribution lists.

Finally, InvolveMe assumes that with its targeting strategy, sponsors, through their well developed competitive intelligence, will be informed about the patient-centric activities of their competitors. Thus InvolveMe expects a viral marketing among its customers regarding its services.

3.5.2.4.4 Social media.

InvolveMe will use social media through InvolveMe Facebook likers and Twitter. Additionally, as some patients' communities have been created by patients on Facebook, InvolveMe will advertise its services on the facebook pages of such

patients' communities. For example, for its first prototype, InvolveMe will advertise its services on the Facebook TTP supporter group (1450 members).

3.5.2.4.5 Website design.

InvolveMe will place a hand-drawn explanation video through an Austrian company called pitch-ink explaining its mission on InvolveMe platform. Following a discussion with the managing director of Pitch-ink, the cost of this company is approximately 5000€.

3.5.2.4.6 Use of Ambassador

Inspired by the story of Paul Watson, pilot of Southwest Airlines, who strikes out to visit the labs of local scientists in the US studying Down syndrome because his 14-year-old son Nathan has the condition, InvolveMe will contact him to be the ambassador of its mission[25].

3.5.2.5 People

To differentiate its services, a science journalist at InvolveMe will assist both researchers in communicating their latest innovations in a jargon free manner and patients in decontextualizing the questions prior to their final submission at InvolveMe platform.

Through a Lab note on its webpage, InvolveMe will update continuously patients and researchers with the outcome of each step related to the crowdsourcing process until the conference date.

InvolveMe makes a customer service agent available to take patients or researchers calls regarding any issue or question when using its platform. Finally, for each rare disease, a customized speech recorded by InvolveMe employees including the contracted postdocs is communicated to the participants during the conferences to share their motivations, activities and challenges faced during the crowdsourcing sessions. The aim of this strategic video is to attenuate the virtual interaction between InvolveMe employees and InvolveMe customers.

3.5.2.6 Physical evidence

Because the conference success depends on the location and the facility layout which can in turn affect the sales quality, the conferences of InvolveMe will be selected in key destinations ensuring at the same time proximity to hotels and airports, proximity to public transport, good parking and good hospitalization system for patients if required.

Access to the conference will be also made available for customers through virtual attendance. Finally, to deliver high quality services to the customers, InvolveMe will outsource to known local experts the conference planning event and the broadcasting activities.

3.5.2.7 Process

As the online registration process on InvolveMe platform constitutes a very important first step that should avoid disengaging participants, InvolveMe will use friendly registration procedures and friendly transactions systems (e.g. PayPal or credit cards).

Being aware of the difficulties that scientists face when communicating their scientific research activities in a jargon free manner to non-scientists, InvolveMe provides scientists with brochures online showing tactics in communicating non-scientific abstracts in a jargon free manner.

The submission of non-scientific abstracts, presentations, patients´ questions and researchers´ answers are performed through InvolveMe webpage in a simplified process via the abstract-, the presentation-, the question- and the answer- submission forms. Furthermore, patients´ questions can be sent by post or E-mail.

Researchers who join InvolveMe conferences can issue by themselves through InvolveMe webpage their certificates of patient centered scientist.

Finally, to be sure that the same standard of service is repeatedly delivered to the customers, InvolveMe will document all created or newly acquired knowledge by using standard operating procedures (SOP) for each part of its service delivery process and update the versions of these SOPs on a routinely basis. By this, knowledge will be quickly and efficiently transferred when InvolveMe scales-up its business.

3.6 Pro Forma Income Statement

Based on the sales projections, InvolveMe will reach profitability in 2019. Figure 9 shows the projected sales, gross profit, and net profit for 2018 up to 2020. Table 9 and 10 summarize all the profits data during the first years of operations for InvolveMe. InvolveMe will stream 90% of its revenues from broadcasting (Figure 12)

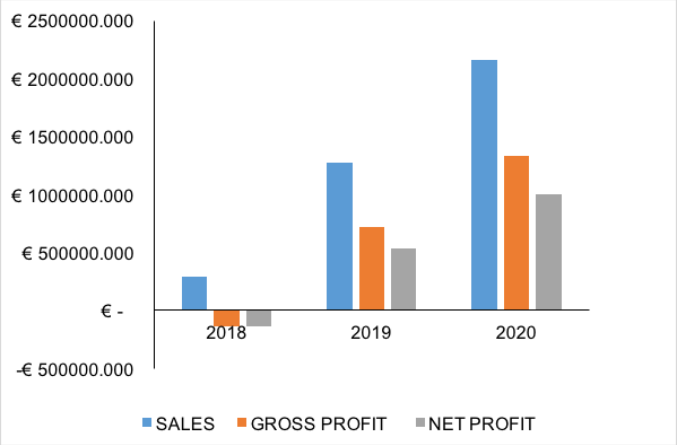


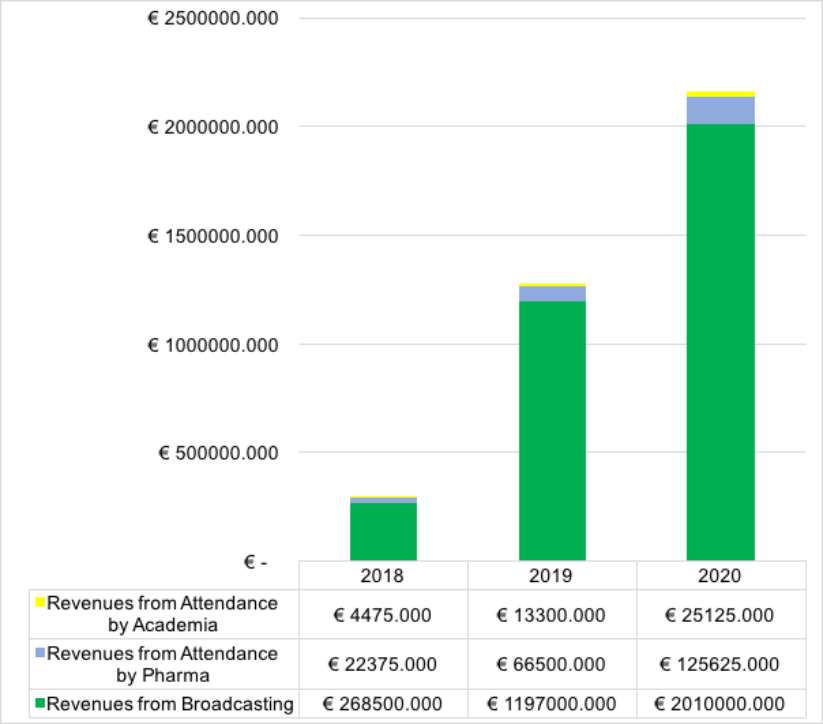
Figure 9. Sales, Gross profit, Net profit 2018-2020, InvolveMe

Table 9. InvolveMe, Pro Forma Income Statement, 2018 (first year) by month (in €)

	Jan.	Feb.	Mar	Apr.	May.	June.	July.	August	Sept.	Oct	Nov.	Dec.	Total
TOTAL REVENUE	-	-	-	26.850	-	-	-	-	-	268.500	-	-	295.350
OPERATING EXPENSES													-
Salaries	14.286	14.286	14.286	14.286	14.286	28.571	14.286	14.286	14.286	14.286	14.286	28.571	200.000
Consultants	35.000					25.000						10.000	70.000
Fringe benefits	4.571	4.571	4.571	4.571	4.571	9.143	4.571	4.571	4.571	4.571	4.571	9.143	64.000
Rent office	4.800	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	18.000
Advertising	15.120	120	120	120	120	120	120	120	120	120	120	120	16.440
Telephone and Internet	200	200	200	200	200	200	200	200	200	200	200	200	2.400
Depreciation of platforms	417	417	417	417	417	417	417	417	417	417	417	417	5.000
Computers, Printer and office accessories	6.175	175	175	175	175	175	175	175	175	175	175	175	8.100
Accounting and payroll	2.550	50	50	50	50	50	50	50	50	50	50	50	3.100
Unexpected expenses	833	833	833	833	833	833	833	833	833	833	833	833	10.000
Video Life streaming	-	-	-	-	-	-	-	-	-	-	-	2.500	2.500
Conference premise	-	-	-	-	-	-	-	-	-	-	-	6.000	6.000
Ticket and stay in Hotel	-	-	-	-	-	-	-	-	-	-	-	1.440	1.440
Legal Setting	25.000	-	-	-	-	-	-	-	-	-	-	-	25.000
TOTAL OPERATING EXPENSES	108.952	21.852	21.852	21.852	21.852	65.709	21.852	21.852	21.852	21.852	21.852	60.649	431.980
GROSS PROFIT (LOSS)	-108.952	-21.852	-21.852	4.998	-21.852	-65.709	-21.852	-21.852	-21.852	246.648	-21.852	-60.649	-136.630

Table 10. InvolveMe, Pro Forma Income Statement, Three years summary (in €)

	Percent	2018	Percent	2019	Percent	2020
TOTAL REVENUE	100,0%	295.350,0	100,0%	1.276.800,0	100,0%	2.160.750
OPERATING EXPENSES		-				-
Salaries	67,7%	200.000,0	24,3%	310.000,0	22,0%	475.500,0
Consultants	23,7%	70.000,0	6,3%	80.000,0	4,4%	95.000,0
Fringe benefits	21,7%	64.000,0	7,8%	99.200,0	7,0%	152.160,0
Rent office	6,1%	18.000,0	1,4%	18.000,0	1,0%	21.600,0
Advertising	5,6%	16.440,0	0,2%	2.160,0	0,1%	3.240,0
Telephone and Internet	0,8%	2.400,0	0,3%	3.600,0	0,2%	5.400,0
Depreciation of platforms	1,7%	5.000,0	0,4%	5.004,0	0,2%	5.004,0
Computers, Printer and office accessories	2,7%	8.100,0	0,5%	5.940,0	0,4%	8.700,0
Accounting and payroll	1,0%	3.100,0	0,2%	3.100,0	0,3%	6.200,0
Unexpected expenses	3,4%	10.000,0	1,2%	15.000,0	1,0%	22.500,0
Video Life streaming	0,8%	2.500,0	0,4%	5.000,0	0,3%	7.500,0
Conference premise	2,0%	6.000,0	0,9%	11.000,0	0,7%	16.000,0
Ticket and hotel	0,5%	1.440,0	0,2%	2.880,0	0,2%	4.320,0
Legal setting	8,5%	25.000,0	-	-	-	-
TOTAL OPERATING EXPENSES	146,3%	431.980,0	43,9%	560.884,0	38,1%	823.124,0
GROSS PROFIT or Loss	-46,3%	-136.630,0	56,1%	715.916,0	61,9%	1.337.626,0
Taxes (25%)	0,0%	-	14,0%	178.979,0	15,5%	334.406,5
NET PROFIT or Loss	-46,3%	-136.630,0	42,1%	536.937,0	46,4%	1.003.219,5



	2018	2019	2020
Revenues from Attendance by Academia	€ 4475.000	€ 13300.000	€ 25125.000
Revenues from Attendance by Pharma	€ 22375.000	€ 66500.000	€ 125625.000
Revenues from Broadcasting	€ 268500.000	€ 1197000.000	€ 2010000.000

Figure 10. Revenue distribution per type of service (2018-2020)

3.7 Pro Forma Cash Flow

Assuming that InvolveMe starts with a capital of €350,000, InvolveMe reaches a positive Cash flow in the second year of its business (2019). With the early bird registrations in April, a first month of positive cash flow is planned followed by another one in October (Figure 11) (Table 11).

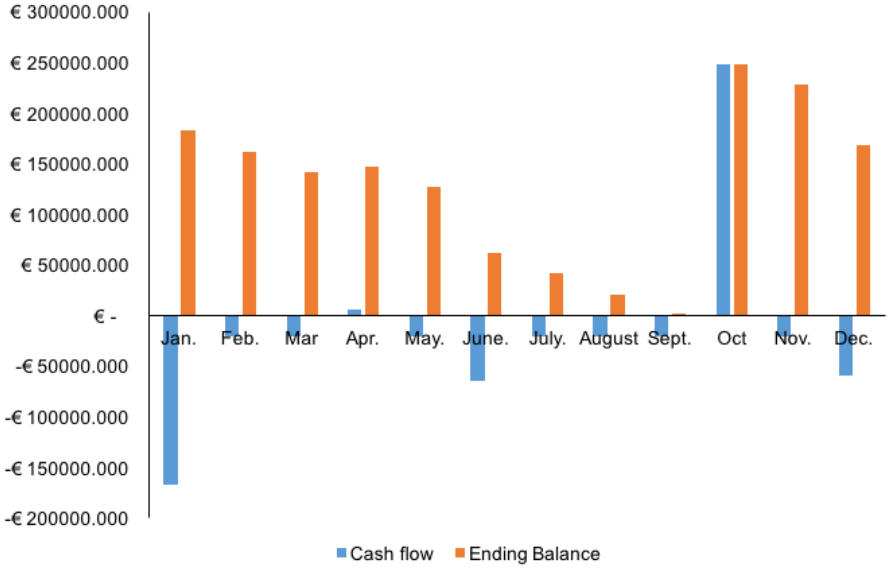


Figure 11. Cash Flow, InvolveMe, 2018

Table 11. InvolveMe, Pro Forma Cash Flow, First Year by Month and 2018-2020 (€)

	Jan.	Feb.	Mar	Apr.	May.	June.	July.	Aug.	Sept.	Oct	Nov.	Dec.	2018	2019	2020
Receipts															
Sales	-	-	-	26.850	-	-	-	-	-	268.500	-	-	295.350	1.276.800	2.160.750
Disbursements															
Salaries	14.286	14.286	14.286	14.286	14.286	28.571	14.286	14.286	14.286	14.286	14.286	28.571	200.000	310.000	475.500
Consultants	35.000	-	-	-	-	25.000	-	-	-	-	-	10.000	70.000	80.000	95.000
Fringe benefits	4.571	4.571	4.571	4.571	4.571	9.143	4.571	4.571	4.571	4.571	4.571	9.143	64.000	99.200	152.160
Rent office	4.800	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	1.200	18.000	18.000	21.600
Advertising	15.120	120	120	120	120	120	120	120	120	120	120	120	16.440	2.160	3.240
Telephone and Internet	200	200	200	200	200	200	200	200	200	200	200	200	2.400	3.600	5.400
Platforms	50.000	-	-	-	-	-	-	-	-	-	-	-	50.000	-	-
Computers and office items	6.175	175	175	175	175	175	175	175	175	175	175	175	8.100	5.940	8.700
Accounting and payroll	2.550	50	50	50	50	50	50	50	50	50	50	50	3.100	3.100	6.200
Unexpected expenses	10.000	-	-	-	-	-	-	-	-	-	-	-	10.000	15.000	22.500
Video Life streaming	-	-	-	-	-	-	-	-	-	-	-	2.500	2.500	5.000	7.500
Conference	-	-	-	-	-	-	-	-	-	-	-	6.000	6.000	11.000	16.000
Ticket and hotel	-	-	-	-	-	-	-	-	-	-	-	1.440	1.440	2.880	4.320
Legal setting	25.000	-	-	-	-	-	-	-	-	-	-	-	25.000	-	-
Total Disbursements	167.702	20.602	20.602	20.602	20.602	64.459	20.602	20.602	20.602	20.602	20.602	59.399	476.980	555.880	818.120
Cash flow	-167.702	-20.602	-20.602	6.248	-20.602	-64.459	-20.602	-20.602	-20.602	247.898	-20.602	-59.399	-181.630	720.920	1.342.630
Beginning Balance	350.000	182.298	161.696	141.094	147.341	126.739	62.280	41.678	21.076	474	248.371	227.769	350.000	168.370	889.290
Ending Balance	182.298	161.696	141.094	147.341	126.739	62.280	41.678	21.076	474	248.371	227.769	168.370	168.370	889.290	2.231.920

3.8 Pro Forma Balance Sheet

A projected balance sheet depicting the position of InvolveMe at the end of 2018 is shown in Table 12.

Table 12. InvolveMe, Pro Forma Balance Sheet, End of 2018 (€)

Assets		
Current assets		
Cash	€ 168.370	
Total current assets		€ 168.370
Fixed assets		
Platforms	€ 50.000	
Less depreciation	€ 5.000	
Total fixed assets		€ 45.000
Total Assets		€ 213.370
Liabilities and owner´s Equity		
Total Liabilities		€ -
Owner´s equity		
Rana Grillberger	€ 175.000	
Partner X	€ 175.000	
Retained earnings	-€ 136.630	
Total Owners equity		€ 213.370
Total Liabilities and owners´ equity		€ 213.370

3.9 Break Even Analysis

The break-even analysis shows that InvolveMe break-even point is at 21 broadcasting orders given a selling price of €15,000 per broadcasted conference, variable costs of €6070 per conference and fixed costs of €183.400 (Figure 12).

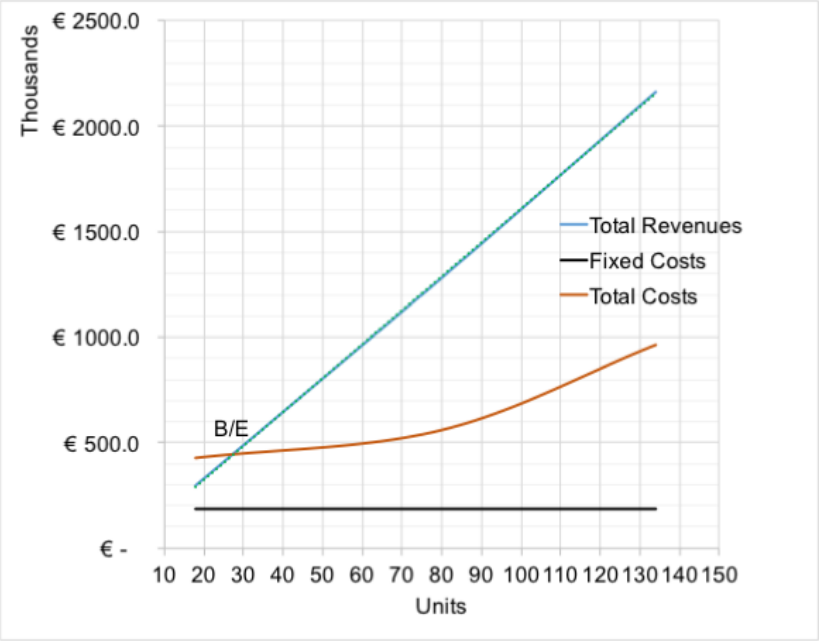


Figure 12. Break even analysis-InvolveMe

3.10 Source of financing

InvolveMe needs a financing of €350,000 to initiate its business. An initial funding will be made by the founder (€175,000) and the remaining €175,000 will be funded either by an interested partner (e.g. the Ludwig Boltzmann Institute) or through a grant from the EU's research and innovation framework program "Horizon 2020 projects". Once a partner joins the founder of InvolveMe, a grant application will be submitted to the European commission within the frame of projects aiming to encourage a stronger discourse between society and science[(26)].

Before the start of the business in January 2018 the following expenses have to be done:

- Legal fees for the license and shareholder contract (€25.000,-)
- 3-month-deposit of the office rent (€3.600,-)

3.11 Organizational Structure

3.11.1 Legal entity

As soon as an interested partner shows interest in InvolveMe business plan, InvolveMe will be founded as an independent owned company in Austria (so-called “Gesellschaft mit beschränkter Haftung Ges.m.b.H”).

3.11.2 Advisory Board

InvolveMe will be accompanied by an advisory board that will be set in 2017.
Advisors that will be contacted are:

- Prof. Kaan Botzug, Medical director at the Ludwig boltzmann institute for rare and undiagnosed-diseases
- Prof. Eva Guinan, Prof Guinan is performing successfully various experiments in Open Innovation at Harvard Medical School
- Prof. Marc Gruber, Ecole Polytechnique Fédérale de Lausanne (EPFL), Chair of Entrepreneurship & Technology Commercialization at EPFL
- Prof Lieke van der Scheer, Faculty of Behavioural, Management & Social Sciences, University of Twente, Enschede, The Netherlands
- Advisor from the NORD (tbd)
- Advisor from EURORDIS (tbd)

3.11.3 Management and functions

In the starting phase, the following organizational structure is foreseen:

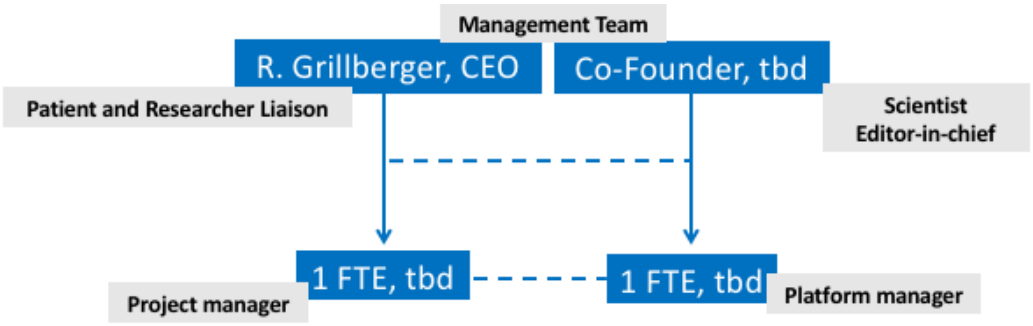


Figure 13. Organizational chart, InvolveMe

Additional tasks will be dedicated to the CEO, the editor in Chief and the platform manager. The CEO will be also in charge of customer service, contracting and human resources. The CV of Rana Grillberger is attached in Appendix 2. The Editor in Chief will be acting as science journalist and will be responsible of public relations. The platform manager will be responsible of IT management.

To avoid high fixed costs in the starting phase, InvolveMe will outsource legal counseling and contract postDocs for preparing a list of key researchers for orphan designated drugs per each rare disease targeted. Accounting and Payroll are also outsourced.

3.11.4 Personnel Plan

Salaries for each employee consist of 14 months (Table 13). Salaries are foreseen to be €50,000/year/employee. In 2017, an interested partner will join the management team. In the same year, one project manager and a platform manager will complete the team. In 2019, after the successful market entrance, a science journalist and another platform manager are hired. In 2020, three other employees will join InvolveMe: a Finance manager, Marketing manager and another Project manager.

InvolveMe is aware of the low salaries paid to its employees. Therefore, InvolveMe will offer to each of its employees a salary increase of 5% per year. Furthermore,

InvolveMe offers two-month sabbatical for employees hired between 2018 and 2020 after three years of employment.

Table 13. Personnel overview (2018-2020), InvolveMe

Personnel Plan	Jan.	Feb.	Mar	Apr.	May.	June.	July.	Aug.	Sept.	Oct	Nov.	Dec.	2018	2019	2020
R. Grillberger, CEO	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 50.000	€ 52.500	€ 55.125
tbd (Scientist Edito-in-chief)	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 50.000	€ 52.500	€ 55.125
tbd (Project Manager)	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 50.000	€ 52.500	€ 55.125
tbd (Plaform Manager)	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 3.571	€ 7.143	€ 50.000	€ 52.500	€ 55.125
tbd (Science journalist)	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ 50.000	€ 52.500
tbd (Plaform Manager)	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ 50.000	€ 52.500
tbd (Finance Manager)	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ 50.000
tbd (Marketing Manager)	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ 50.000
tbd (Project Manager)	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ -	€ 50.000
Total People	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**4**	**6**	**9**
Total Payroll	€ 14.286	€ 14.286	€ 14.286	€ 14.286	€ 14.286	€ 28.571	€ 14.286	€ 14.286	€ 14.286	€ 14.286	€ 14.286	€ 28.571	€ 200.000	€ 310.000	€ 475.500

4 SURVEY RESULTS

4.1 Survey among researchers

22 researchers from the public and private sectors researching thrombotic thrombocytopenic purpura (TTP) that is foreseen to be the first prototype of InvolveMe were invited by E-mail to answer two questions for various service features of InvolveMe. For each service feature a pair of functional and dysfunctional question was formulated to which the researcher can answer in one of five different ways according to the Kano theory (Appendix 3). Out of 22, only 6 researchers took the survey: 5 were key opinion leaders from academia and medical centers researching TTP and 1 R&D director from the pharmaceutical industry. Despite the very small number of participants, the results were evaluated using the Kano evaluation method. Pair of points referring to the contribution to satisfaction or dissatisfaction for each service feature were plotted on a two dimensional representations of Kano quality categories (Figure 14). No “attractive” features for the customers were identified.

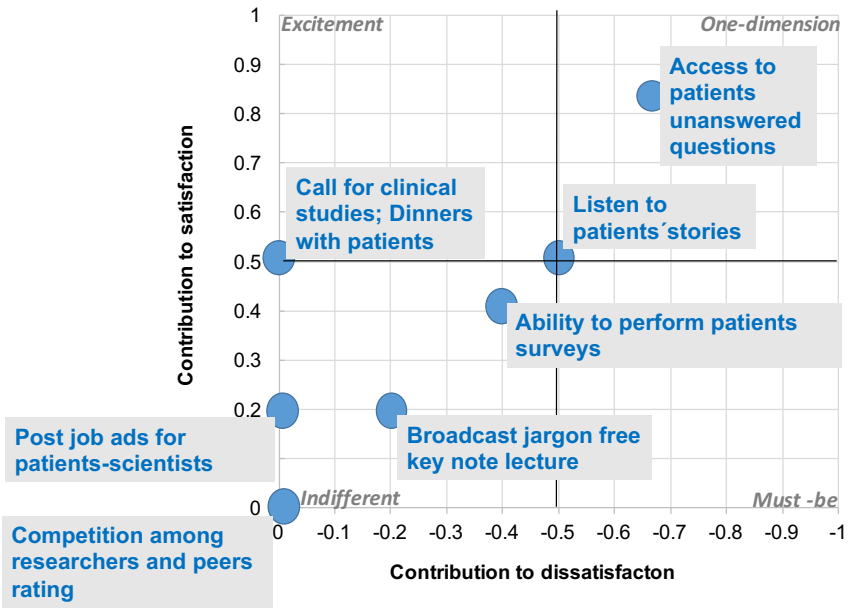


Figure 14. Customer requirement categories - Kano Model

However, three conclusions can be driven from figure 14. First, the feature “access to patients unanswered questions” (XY pair: -0.7; 0.8) being in the upper right quadrant can be viewed as a *one-dimension* attribute. Hence it is the most worth including requirement for InvolveMe customers. Second, customers are *indifferent* toward the feature “competition among researchers to answer patients´ questions and peer-peer rating” (XY pair: 0;0), therefore this service feature can be left out. Finally, the feature “ability to perform patients´ surveys” (XY pair: -0,4; 0,4), “broadcasting jargon free key note lectures by key opinion leaders” (XY pair: 0,2; 0,2), and “placing job ads to recruit patients-scientists” (XY pair: 0; 0.2) are spaced in the *Indifferent* category indicating that they are not needed by the customer; however, this may change over time to one dimension or excitement. The feature “Call for clinical studies” (XY pair: 0; 0.5), “organized dinners with patients” (XY pair: 0; 0.5), “listening to patients´ stories” (XY pair: -0.5; 0.5) are fuzzier results because they are at the intersection of several categories. Hence, it could reflect a disagreement among respondents.

To understand the relative importance of each feature of InvolveMe services from the researchers´ perspective, a self stated importance questionnaire was also included in the survey. The respondents´ average importance assigned to each question differed between academia (N=5) and the pharmaceutical industry (N=1) (Figure 15): i) “Presenting jargon free conferences to patients” was scored as “very important” for the researcher from the pharmaceutical industry while “only somewhat important” for researchers from academia. The result of the academia is not surprising because very likely researchers consider that they have previously been perfectly capable of doing research without patient involvement. Furthermore, involving patients costs time and money. Next, financing structures and procedures do not always encourage patient involvement ii) The researcher from the pharmaceutical industry assigned extreme importance (score 5) to having access to a list of patients´ unanswered questions while the same feature was considered as only important (average score 2.8) for researchers from the public sector. This difference is logical as academia and medical centers are much more in contact with patients compared to the pharmaceutical industry iii) Both groups assigned “the ability to call patients during the conference for clinical studies” as important, and this can be explained by the fact that patients with rare diseases, due to their low number, cannot be easily found to participate in clinical studies. Last iv) all respondents considered “competing among researchers to answer

the patients´ questions" and "having their answers peer to peer rated" as non important to somewhat important. This result correlated clearly with the coordinates of these two requirements in Figure 14.

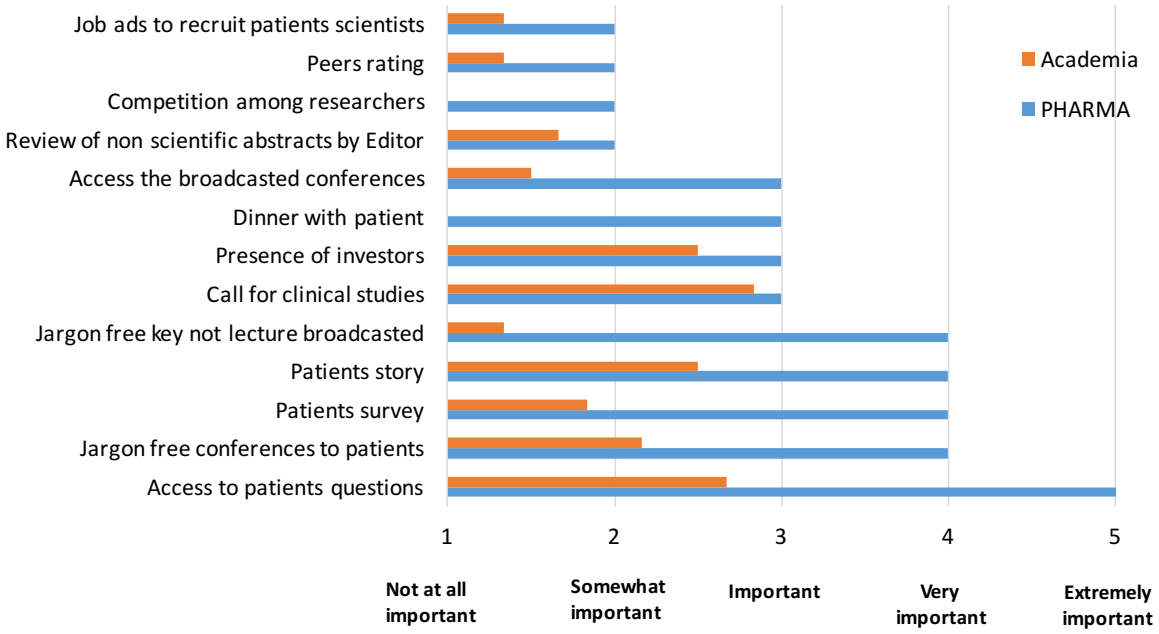


Figure 15. Self statement importance average- Kano Model

4.2 Survey among patients

A Kano questionnaire was not sent to patients as the business idea of InvolveMe is derived from the results of a previous survey that was done earlier in 2016 with 78 patients with TTP through the "TTP supporters group" with 1500 members on Facebook. The survey revealed that most of the patients are well informed about their disease however, 65% of the respondents would like to learn about advances in research to treat their rare disease and 95% would like to share their TTP stories with researchers in TTP (Figure 16).

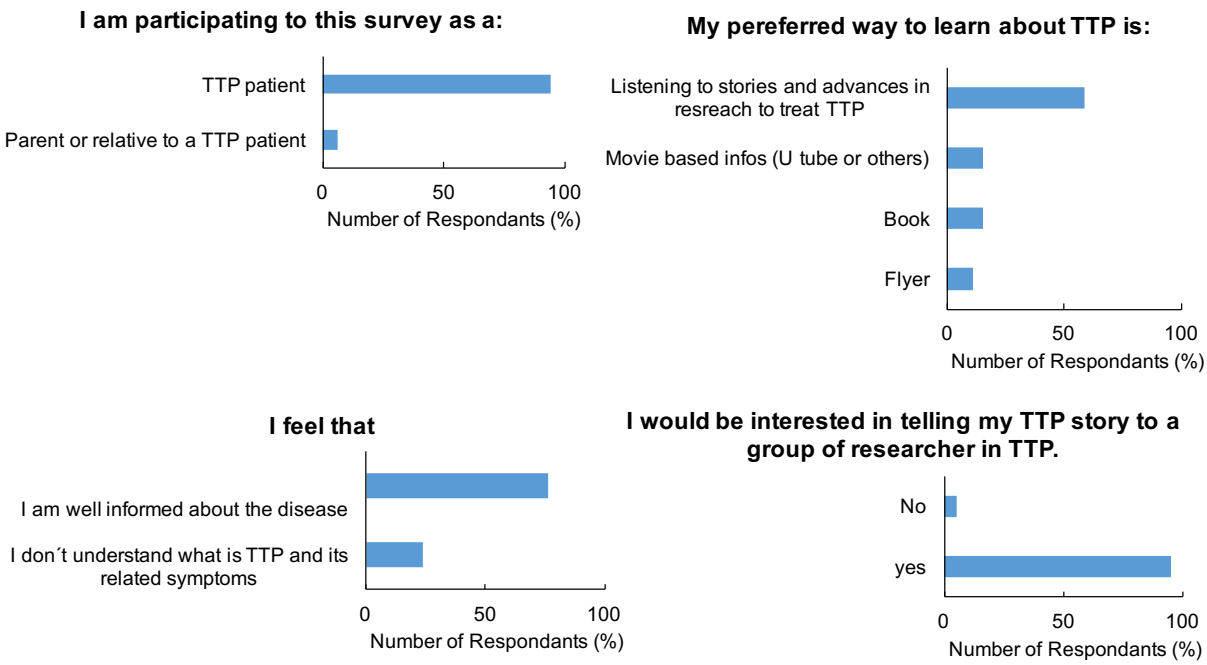


Figure 16. Survey results - TTP Patients

5 CONCLUSION

This thesis aimed to answer three main research questions and to set a business plan for InvolveMe. I feature below the answers to the research questions raised and include few proposals for future research.

Question 1. What are the known characteristics of services in general and how to set characteristics for InvolveMe services to improve the customer experience when using them?

The known characteristics of services in the literature are Intangible, Heterogeneous, Inseparable and Perishable (IHIP). Scholars recommend to attenuate these characteristics. The IHIP characteristics described were partially applicable to InvolveMe. For instance, InvolveMe services are intangible and heterogeneous but not necessarily inseparable and perishable. InvolveMe customers can view non-scientific abstracts and patients´ questions on its platforms prior to the conference date. Furthermore, they can attend virtually its conferences and do not need to be physically present at the location where the conferences are performed.

To influence the intention of customers to use InvolveMe services, three service features were adjusted to decrease their intangibility and heterogeneity. The first one is to let scientists issue by themselves through the webpage of InvolveMe their certificates of “patient centered scientist” increasing by this the tangibility of InvolveMe services. Second, to give online access to the non-scientific abstracts and questions/answers presented at the conference. The third one is to let science journalist edit all documents submitted by InvolveMe customers to deliver a standard quality of jargon free abstracts, presentations and answers to patients.

Question 2. What marketing concepts should InvolveMe formulate as a service provider to attract attendees and increase the number of participants?

To attract attendees and increase the number of participants, InvolveMe has to use the 7Ps services mix approach (Product, Price, Place, Promotion, People, Physical evidence, Processes). *Product* refers to InvolveMe services where patients learn from scientists about their latest innovations and where researchers get access to unanswered patients´ questions. *Price* is dominated by the registration and

broadcasting fees. *Place* refers to the access to InvolveMe crowdsourcing platforms and to the conferences venue be it live or virtual. *Promotion* encompasses partnering with patients support organization, back-riding on sales channels of lab materials suppliers, the use of an ambassador, phone calls and social media to provide customers information with InvolveMe offerings. *People* includes the live and virtual interaction between customers and InvolveMe employees like the science journalists and customer service agents. *Physical evidence* is the key destination where the conference is organized. Finally, *Process* refers to the use of simple registration procedures online, friendly platforms and a strong organizational learning within the organization of InvolveMe.

Question 3. Finally, how should InvolveMe design its new services in a way that delights customers?

To design a new service that delight customers, the Kano questionnaire was employed where customer requirements were anticipated. No “attractive” features for the customers were identified. This result is very likely due to the newness of such services. However, the answers to this questionnaire showed that InvolveMe must focus on delivering a large number of high quality patients´ questions to the researchers´ community due to the importance of this requirement to them. Furthermore, although not strongly indicative of an attractive customer requirement at the moment, InvolveMe should allow researchers to invite patients to participate in their clinical studies during the conferences. Finally, InvolveMe has to encourage patients to share their stories with the researchers´ community as this feature scores as important for both pharma and academia. Despite the low number of participants who took the survey, the results of this questionnaire increased my understanding of what attract researchers the most when using InvolveMe services. The addition of the self statement importance was very much helpful to discriminate among the requirements of public and private sectors. I also noted various observations with the Kano method. Writing the double questions in the functional and dysfunctional format was a very difficult task as I had to abstract the content of each service feature to come up with a short question. Regarding the functional questions, the wording confused and disengaged participants who tested the survey before its finalization. The confusion was between “I like it that way” and “It must be that way”. Therefore, for

services, I had to use alternative wording like “Wow I like it” and “No big reaction, I expect it of course” to differentiate better among the responses.

Future research suggests to compare various characteristics of contemporary services involving communities´ co-creation and check whether the IHIP characteristics are still a valid framework for services. Additionally, it would be interesting to measure the influence of each element of the 7Ps on the users´ intention to use services involving communities´ co-creation.

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APPENDICES

Appendix 1. List of sponsors per designated orphan indication

Designated Orphan Indication	Sponsor
Treatment of thrombotic thrombocytopenic purpura	Ablynx
	Omeros
	Shire
	Glenmark Pharmaceuticals
Treatment of acute myeloid leukaemia	AbbVie Ltd
	Aprea AB
	Aqualis ASA
	Astex Therapeutics Limited
	Boehringer Ingelheim International GmbH
	Bristol-Myers Squibb Pharma EEIG
	Celator (UK) Ltd
	Celgene Europe Limited
	Cyclacel Limited
	Daiichi Sankyo Development Ltd
	DCPrime BV
	Diamond BioPharm Limited
	Diatos S. A.
	Dr Ulrich Granzer
	EleosInc Limited
	ERYtech Pharma S.A.
	Fate Therapeutics, LTD
	Hybrigenics SA
	IPD - Therapeutics BV
	Janssen-Cilag International NV
	Kanisa Europe Limited, Mofo Notices Limited, C/Morrison & Foerster MNP
	Karyopharm Europe GmbH
	Kiadis Pharma Netherlands B.V.
	LTKFarma
	Mapi Ireland Limited
	Meda AB
	Merck KGaA

Treatment of acute myeloid leukaemia (Continued)	Novartis Europharm Limited
	Orsenix Holdings BV
	Otsuka Pharmaceutical Europe Ltd.
	Pfizer Limited
	Pierre Fabre Médicament
	Regulatory Resources Group Ltd
	Roche Registration Limited
	Seattle Genetics UK, Limited
	SELLAS Life Sciences Group UK, Limited
	Spector Consulting SAS
	Sunesis Europe Ltd
	The Chancellor, Masters and Scholars of the University of Oxford
	Theorem Clinical Research GmbH
	TMC Pharma Services Ltd
	Vion (UK) Limited, % i3 Research
	Voisin Consulting S.A.R.L.
	Xenetic Biosciences Plc
Treatment of glioma	AbbVie Ltd
	Activartis Biotech GmbH
	Alan Boyd Consultants Ltd
	Antigenics Therapeutics Limited
	Apogenix GmbH
	Avena Therapeutics Ltd
	Biocompatibles UK Limited
	Clinical Network Services (UK) Ltd
	CytoVac A/S
	Diamond BioPharm Limited
	Dr Matthias Luz
	Dr Regenold GmbH Development·Regulatory·Market Access
	DualTpharma B.V.
	Eisai Europe Limited
	Eli Lilly Nederland B.V.
	Envigo Pharma Consulting Ltd

Treatment of glioma (Continued)	Epitarget AS
	ERC Belgium
	EUDRAC Limited
	FLAG Therapeutics Ltd
	GW Research Ltd
	IDIS Ltd
	Intsel Chimos SA
	Isabelle Ramirez
	Lipopharma Therapeutics SL
	Northwest Biotherapeutics GmbH
	Noxxon Pharma AG
	Oligovax
	Oncoscience AG
	Orphit
	Orphix Consulting GmbH
	Prof. Olivier Blin
	Sigma-Tau Industrie Farmaceutiche Riunite S.p.A
	STEMGEN S.p.A
	Therapeia GmbH & Co. KG
	Virttu Biologics Limited
Treatment of cystic fibrosis	Alaxia
	AlgiPharma AS
	AOP Orphan Pharmaceuticals AG
	Aradigm Limited
	Arch Bio Ireland Ltd
	Bayer Pharma AG
	Biological Consulting Europe Ltd
	Clinical Network Services (UK) Ltd
	Coté Orphan Consulting UK Limited
	CSL Behring GmbH
	CURx Pharma (UK) Limited
	Erydel S.p.A.
	Grifols Deutschland GmbH
	Immunsystem I.M.S. AB
	Imperial Innovations Limited

Treatment of cystic fibrosis (Continued)	Istituto Europeo per la Ricerca sulla Fibrosi Cistica - ONLUS
	LABORATOIRES SMB SA
	Lamellar Biomedical Ltd
	Merck Sharp & Dohme Limited
	Meristem Therapeutics S.A.
	Mucokinetica Ltd
	NovaBiotics Ltd
	Novoteris
	Ockham Biotech Limited
	PD Dr med. Joachim Riethmüller
	Pharm Research Associates (UK) Limited
	Pharmaxis Pharmaceuticals Limited
	Plexcera Therapeutics EU Limited
	ProQR Therapeutics III BV
	ProtAffin Biotechnologie AG
	PTC Therapeutics International Limited
	Rare Partners srl Impresa Sociale
	Synovo GmbH
	Triskel EU Services Ltd
	Vertex Pharmaceuticals (Europe) Limited
Treatment of pancreatic cancer	AB Science S.A.
	Aduro Biotech Holdings, Europe B.V.
	Baxalta Innovations GmbH
	Cato Europe GmbH
	Dr Ulrich Granzer
	ERYtech Pharma S.A.
	Eurogen Pharmaceuticals Limited
	European Medical Advisory Services Limited
	GANYMED Pharmaceuticals AG
	Gemvax A/S
	ICON Clinical Research (U.K.) Limited
	Immodulon Therapeutics Ltd
	Immunomedics GmbH
	Isarna Therapeutics GmbH

Treatment of pancreatic cancer (Continued)	Kadmon International Ltd
	Karcinolys S.A.S
	Lokon Pharma AB
	MBiotec GmbH
	MediGene AG
	Merck KGaA
	Oncolytics Biotech (UK) Limited
	Oncoscience AG
	Pharm Research Associates (UK) Limited
	Pharmacyte Biotech Europe Limited
	Raptor Pharmaceuticals Europe BV
	Regulon AE
	RESprotect GmbH
	Synovo GmbH
	Targovax AS
	Theradex (Europe) Ltd.
	VCN Biosciences S.L.
Treatment of ovarian cancer	AbbVie Ltd
	Æterna Zentaris GmbH
	Amgen Europe B.V.
	Aprea AB
	ASPHALION, SL
	AstraZeneca UK Limited
	Clovis Oncology UK Limited
	Diamond BioPharm Limited
	Eisai Europe Limited
	Endocyte Europe B.V.
	Galileo Research S.r.l.
	GANYMED Pharmaceuticals AG
	ICON Clinical Research (U.K.) Limited
	ImmunoGen Europe Limited
	Merck Sharp & Dohme Limited
	Nektar Therapeutics UK Ltd
	Neovii Biotech GmbH

Treatment of ovarian cancer (Continued)	Oasmia Pharmaceutical AB
	Oncolytics Biotech (UK) Limited
	Pharma Mar S.A.
	Prima Biomed GmbH
	PsiOxus Therapeutics Ltd
	Right Track Regulatory Limited
	Takeda Development Centre Europe Ltd
	Targovax Oy
	TESARO U.K. Limited
	TMC Pharma Services Ltd
	ViRexx International Corp. Limited
Treatment of chronic lymphocytic leukaemia	AbbVie Ltd
	Acerta Pharma, BV
	Amgen Europe B.V.
	BlackSwan Pharma GmbH
	Celgene Europe Limited
	EleosInc Limited
	Emergent Product Development UK Limited
	Hybrigenics SA
	Immunomedics GmbH
	Janssen-Cilag International NV
	Karyopharm Europe GmbH
	Kite Pharma UK, Ltd
	LFB-Biotechnologies
	Lymphact - Lymphocyte Activation Technologies S.A.
	Merck Sharp & Dohme Limited
	MorphoSys AG
	Mundipharma Research Limited
	Novartis Europharm Limited
	Roche Registration Limited
	Theorem Clinical Research GmbH
Treatment of hepatocellular carcinoma	4SC AG
	Bayer Pharma AG
	Biological Consulting Europe Ltd

Treatment of hepatocellular carcinoma (continued)	Daiichi Sankyo Development Ltd
	Dr Francesco Izzo
	Dr Ulrich Granzer
	Eisai Europe Limited
	Eli Lilly Nederland B.V.
	Interface International Consultancy Limited
	Kowa Pharmaceutical Europe Co. Ltd
	MolMed S.p.A.
	Nerviano Medical Sciences Srl
	New B Innovation (UK) Limited
	PBS Regulatory Consulting Group Limited
	S-cubed Ltd
	SciClone Pharmaceuticals Italy S.r.l
	TLC Biopharmaceuticals B.V.
	Transgene S.A.
Treatment of acute lymphoblastic leukaemia	Amgen Europe B.V.
	ARIAD Pharma Ltd
	Bristol-Myers Squibb Pharma EEIG
	ERYtech Pharma S.A.
	EUSA Pharma SAS
	Fate Therapeutics, LTD
	Kite Pharma UK, Ltd
	LTKFarma
	Napp Pharmaceuticals Research Limited
	NDA Regulatory Science Ltd
	Nova Laboratories Limited
	Novartis Europharm Limited
	Only for Children Pharmaceuticals
	Orbona Pharma Ltd
	Sigma-Tau Rare Diseases, S.A.
	Voisin Consulting S.A.R.L.
	Xenetic Biosciences Plc
Treatment of Duchenne muscular dystrophy	AVI BioPharma International Ltd
	Biological Consulting Europe Ltd
	BioMarin International Limited

Treatment of Duchenne muscular dystrophy (continued)	Envigo Pharma Consulting Ltd
	EUDRAC Limited
	FGK Representative Service GmbH
	Généthon
	Italfarmaco S.p.A.
	Karl Rouger
	N-GENE Kutatási és Fejlesztési Kft
	NicOx
	Pfizer Limited
	PTC Therapeutics International Limited
	ReveraGen BioPharma Limited
	Santhera Pharmaceuticals (Deutschland) GmbH
	Summit (Oxford) Limited
	uniQure biopharma B.V.
Treatment of amyotrophic lateral sclerosis	Brainstorm Cell Therapeutics UK Ltd
	California Stem Cell (UK) Ltd
	Dr Regenold GmbH Development·Regulatory·Market Access
	Herantis Pharma Plc
	Knopp Neurosciences Sub Ltd
	Mitsubishi Tanabe Pharma Europe Ltd.
	NeuroVision Pharma GmbH
	Newron Sweden AB
	Orphazyme ApS
	PBS Regulatory Consulting Group Limited
	Pharma Gateway AB
	QRC Consultants Ltd
	Shore Limited
	Treeway B.V.
	University of Sheffield
Treatment of retinitis pigmentosa	Alan Boyd Consultants Ltd
	Clinipace GmbH
	Dompé farmaceutici s.p.a.
	Enpharma Ltd
	HORAMA SAS

Treatment of retinitis pigmentosa (continued(	Nanovector s.r.l.
	Natac Pharma S.L.
	ProRetina Therapeutics S.L.
	QLT Ophthalmics (UK), Ltd
	ReNeuron Ltd
	Sanofi-Aventis groupe
	Shire Pharmaceuticals Ireland Limited
	TMC Pharma Services Ltd
	Universitätsklinikum Tübingen (UKT)
	Voisin Consulting S.A.R.L.
Treatment of mucopolysaccharidosis (Sponsor cover distinct types)	Alexion Europe SAS
	Axcentua Pharmaceuticals AB
	BioMarin Europe Ltd.
	Cochamo Systems Ltd
	Dr Ulrich Granzer
	Fondazione Telethon
	Institut Pasteur
	Laboratorios del Dr Esteve, S.A.
	LYSOGENE
	Plexcera Therapeutics EU Limited
	PTC Therapeutics International Limited
	Shire Human Genetic Therapies AB
	Shire Pharmaceutical Development Limited
	Ultragenyx UK Limited
	Voisin Consulting S.A.R.L.
Treatment of cutaneous T-cell lymphoma	Actelion Registration Ltd
	Allos Therapeutics Limited
	AOP Orphan Pharmaceuticals AG
	Celgene Europe Limited
	Clinipace GmbH
	Eisai Limited
	ExperGen Drug Development GmbH
	Galderma R&D
	Innate Pharma S.A.
	Kinesys Consulting Ltd

Treatment of cutaneous T-cell lymphoma (continued)	Napp Pharmaceuticals Research Limited
	Takeda Pharma A/S
	TenX Biopharma Ltd
	Winston Laboratories Ltd
Treatment of systemic sclerosis	Active Biotech AB
	arGentis Autoimmune Europe Limited
	Assistance Publique Hôpitaux de Marseille
	Bayer Pharma AG
	Covis Pharma S.à.r.l.
	Cytori Ltd
	Digna Biotech S.L.
	FGK Representative Service GmbH
	GenKyoTex Innovation S.A.S
	Inventiva
	PPD Global Ltd
	Sanofi-Aventis groupe
	Serodapharm GmbH
	United Therapeutics Europe Ltd
Treatment of haemophilia	Alnylam UK Limited
	Apitope International NV
	Baxalta Innovations GmbH
	Bayer Pharma AG
	Biogen Idec Limited
	CSL Behring GmbH
	Novo Nordisk A/S
	Pharma Gateway AB
	Richardson Associates Regulatory Affairs Ltd
	Roche Registration Limited
	uniQure biopharma B.V.

Appendix 2. Founder´s CV

Rana Grillberger

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E-Mail: grillberger.rana@gmail.com

Key Competencies

- ➢ Thorough understanding of open innovation strategies for generating inputs to early stage innovation processes
- ➢ Expertise in immunoassays and biochemical analysis techniques with a focus on recombinant proteins and autoantibodies
- ➢ Development of thrombosis and endothelial cells adhesion assays based on static and microfluidic technologies
- ➢ Development of master validation plans related the manufacturing of recombinant proteins according to GMP

Professional Experience

02/2006-09/2015 **Baxalta (Vienna/Austria)**
PhD student / Postdoc in Global Exploratory and Preclinical R&D

- Managed and coordinated external and internal biochemical characterization activities including surface plasmon resonance, N-terminal sequencing, peptide mapping, antibody profiling, Fourrier Transform Infra-Red Spectroscopy (FTIR) and dynamic light scattering (DLS)

- Developed cell adhesion assays based on a microfluidic technology using an automated platform to study under flow conditions the activity of enzymes in the presence or absence of specific autoantibodies. The developed assay allowed the characterization of individual preclinical and clinical phase I batches of recombinant enzymes

09/2003-01/2006 **Baxalta (Neuchâtel/Switzerland)**
Process validation engineer

- Wrote and reviewed master validation plans, wrote and executed protocols, wrote reports related to the validation of cleaning processes following the manufacturing of recombinant FVIII

- Developed strategies and risk assessment based approaches using risk analysis tools for cleaning processes to ensure drug agencies approval

Education

04/2016-07/2017	**Lab for open innovation in science - LOIS** **Ludwig Boltzmann Institute** Education program aiming to apply open Innovation methods to drive successful collaborations between various stakeholders including academia, industry and patients organizations
10/2014-06/2016	**MBA-Entrepreneurship and Innovation** Vienna University of Economics and Business, Vienna, Austria.
2007/2014	**Dr. rer. Nat. tech. (Ph.D.)** / Grade: pass with distinction Specialization: Biotechnology University of Natural Resources and Applied Life Sciences, Vienna, Austria

Patent

Leopold Grillberger, Alexandra Spenger, Meinhard Hasslacher, **Rana Grillberger**, Manfred Reiter. 2014. Cell culture medium for ADAMTS protein expression. WO/2011/014838A1.

Publications

Rana Grillberger,Veronica C. Casina, Peter L. Turecek, X. Long Zheng, Hanspeter Rottensteiner, and Friedrich Scheiflinger. (2014). Anti-ADAMTS13 IgG autoantibodies present in healthy individuals share linear epitopes with those in patients with thrombotic thrombocytopenic purpura. Haematologica. 99(4):e58-60.

R. Grillberger, B. Gruber, S. Skalicky, G. Schrenk, P. Knöbl, B. Plaimauer, P. L. Turecek, F. Scheiflinger and H. Rottensteiner. A novel flow-based assay reveals discrepancies in ADAMTS13 inhibitor assessment as compared with a conventional clinical static assay. (2014). Journal of Thrombosis and Haemostasis. 12: 1523–1532

Benhamou Y1, Assié C, Boelle PY, Buffet M, **Grillberger R**, Malot S, Wynckel A, Presne C, Choukroun G, Poullin P, Provôt F, Gruson D, Hamidou M, Bordessoule D, Pourrat J, Mira JP, Le Guern V, Pouteil-Noble C, Daubin C, Vanhille P, Rondeau E, Palcoux JB, Mousson C, Vigneau C, Bonmarchand G, Guidet B, Galicier L, Azoulay E, Rottensteiner H, Veyradier A, Coppo P. (2012). Development and validation of a predictive model for death in acquired severe ADAMTS13 deficiency-associated idiopathic thrombotic thrombocytopenic purpura: the French TMA Reference Center experience. *Haematologica. 97(8):1181-6*

Language skills

English (Proficient), French (Proficient), German (Operational Proficiency), Arabic (Mother Tongue)

Citizenship

Austrian and Lebanese

Appendix 3. Kano Questionnaire sent to researchers

Introduction

InvolveMe is an open innovation project explored in my MBA thesis. It aims to bridge patients with rare diseases to researchers and to transit patients from the role of consumers to participants. The first disease that InvolveMe will cover is Thrombotic Thrombocytopenic Purpura.

What is InvolveMe exactly? InvoveMe is a project that aims to invite researchers from Academia and Pharmaceutical companies researching a specific rare disease to share in a conference their jargon free innovation stories with patients affected by the same rare disease. The abstracts are submitted by scientists for consideration by the patients according to their level of interest- ant not by a scientific committee. The company offers scientists assistance by editors who are expert in communicating non scientific abstracts to the public.

InvolveMe invites also patients affected by a specific rare disease to submit anonymously a question that researchers may be able to answer and then brings researchers and physicians together through a crowdsourcing platform to formulate answers to the questions deemed relevant by the patient´s community.

You decide to register and submit your abstract. Assume you receive a notification by E-mail from InvolveMe that your abstract was selected by the patients for an oral presentation. Please answer the questions of this survey.

Note: Some questions may appear redundant but are asked intentionally to address customer needs, please don´t be annoyed.

Thank you for taking this survey!

Please specify your occupation:

- Health care professional
- Researcher from the pharmaceutical industry
- Academic
- Medical resident
- PhD Student
- Post do

How would you feel if the following features are included in the service of InvolveMe?					
	Wow, I like it	**No big reaction. I expect this of course**	**I do not really care**	**I can tolerate it**	**I dislike it**
You can invite patients during your oral presentation to participate in one of your clinical studies					
Prior to submission, you get your non-scientific abstract reviewed by experts in editing jargon free abstracts					
Investors are present in the audience					
You have access to a list of unanswered questions raised by a group of patients with the rare disease you research					
You can enter a competition among researchers online to answer the questions of patients					
You can rate anonymously your peers´ answers to the questions raised by the patients. Researchers whose answers score the best are awarded.					
You can advertise job ads on InvolveMe platform aiming to recruit scientists who are also patients affected by the disease you research to join your organization					
You can make patients survey through InvolveMe					
You can have a dinner with patients affected by the rare disease you research.					
You can listen to a patient´s story regarding his/her journey with a rare disease					
InvolveMe conferences can be broadcasted and no need to attend them in person.					
You can broadcast a jargon free key note lecture held by a key opinion leader to employees in your organization					

How would you feel if the following features are <u>not included</u> in the service of InvolveMe?				
	I like that this feature is absent	**I would not really care**	**A pity, but I can live without this feature**	**I don´t like that this feature is absent**
Ability to invite patients during your oral presentation to participate in one of your clinical studies				
Review of your non-scientific abstract by experts in editing jargon free abstracts prior to submission				
Presence of Investors in the audience				
Access to a list of unanswered questions raised by a group of patients with the rare disease you research				
Ability to enter a competition among researchers online to answer questions raised by patients				
Ability to rate anonymously your peers´ answers to the questions raised by the patients.				
Job ads posting on InvolveMe platform aiming to recruit scientists who are also patients affected by the disease you research to join your organization				
Ability to make patients´ survey through InvolveMe.				
Organized dinners with patients affected by the rare disease you research				
Communication of a patient´s story regarding his/her journey with a rare disease				
Broadcasting of InvolveMe conferences				
Broadcasting of a jargon free key note lecture held by a key opinion leader to employees in your organization				

How important is it for you to:					
	Not at all important	**Somewhat important**	**Important**	**Very important**	**Extremely important**
Present orally non scientific abstracts to patients affected by a rare disease you research?					
Be able to invite patients to participate in one of your clinical studies?					
Be assisted by editors to write jargon free abstracts to the public?					
Communicate to investors your current research activities to motivate them to invest in your research project?					
Access a list of unanswered questions raised by a group of patients with the rare disease you research?					
Enter a competition among researchers online to answer questions raised by patients living with the rare disease you research?					
Rate your peers´ answers to the questions raised by the patients?					
Hire a scientist who is also a patient affected by the rare disease you research to join your organization?					
Make patients survey?					
Have a dinner with patients affected by the rare disease you research?					
Listen to a patient´s story living with the rare disease your research?					
Get non scientific conferences broadcasted instead of attending them in person?					