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How to improve the diagnosis and treatment of rare diseases?

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During the preparation of this master's thesis, the author(s) utilised ChatGPT and Grammarly for the following purposes:

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By signing this declaration, we affirm that the content of this master's thesis reflects our original work, augmented by the responsible use of AI.

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Jade Spineux

Foreword:

I would like to thank all the people who contributed and helped me during the writing of this thesis, directly or indirectly.

I would like to thank my supervisor, Mr. Paul Belleflamme, for his valuable guidance and support throughout the writing of the thesis. His comments and advice significantly improved my work.

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Introduction

Rare diseases affect between 350 and 475 million people globally, according to the estimation of the World Economic Forum. Approximately half of those people are children. Those numbers pose extraordinary challenges for patients, caregivers and the healthcare system (Adachi et al., 2023). Although individually uncommon, these diseases collectively impact millions, yet more prevalent conditions often overshadow them in terms of research funding, public awareness, and policy focus. This causes severe difficulties for patients, such as delays and challenges in accessing adequate diagnosis, creating prolonged diagnosis journeys; they can also encounter insufficient access to specialised care, and as a result, this can cause high economic burdens on their families but also on society. This lack of attention to rare diseases causes a slowdown in research and innovation and thus creates a cycle of underdiagnosis and inadequate treatment. (Adachi et al., 2023) (Stoller, 2018)

Patients with rare diseases face a diagnostic odyssey, they frequently have to consult several healthcare providers over several years before receiving an accurate diagnosis. During this period, patients may encounter multiple misdiagnoses. Even after diagnosis, barriers to accessing treatment remain pervasive, particularly in low-resource settings where healthcare systems struggle to prioritise rare conditions. Rare diseases also have a substantial financial burden since direct medical casts often outweigh the household income. This specific issue exacerbates even more the psychological and social toll. (Adachi et al., 2023) (Stoller, 2018)

Researchers and clinicians also face unique challenges due to the rarity of these conditions. Indeed, assembling sufficiently large patient cohorts for studies and research and gathering enough data is difficult. The high research and development costs limit the availability of effective treatment. While patient advocacy groups have made significant strides in fostering collaboration among stakeholders, substantial knowledge, funding, and infrastructure gaps persist.

Innovative methods that close the gap between patients and researchers are desperately needed in the face of these obstacles. This thesis suggests the development of a two-sided digital platform that would connect these groups, allowing researchers to obtain anonymised datasets essential for deepening rare disease research and patients to exchange their data safely. By addressing the core challenges of trust, accessibility, and collaboration, this platform seeks to accelerate diagnosis, improve research, and boost treatment results.

This thesis aims to provide a comprehensive business plan for the proposed platform, it covers its development, launch, and long-term sustainability. This research presents actionable strategies for creating and capturing value, drawing from various academic and industry sources. The analysis is structured into three key sections:

1. **Uncover Value**: This first section will identify participants' and investigators' needs and behaviours while highlighting the network effect and how it influences the platform adoption.
2. **Create Value**: This second section will define the platform's value mission and value proposition. It will be supported by a competitive analysis and strategies for sustaining user engagement.
3. **Capture Value**: This third section will outline the implementation of a Minimum Viable Platform (MVP), the monetisation model, and the financial projections to guarantee scalability and impact.

Methodology

This section outlines the methodology employed in this thesis. As introduced in the introduction, three main chapters structure the work, each focusing on distinct but interconnected objectives, which will be introduced below. The overall methodology of the thesis is a research-based methodology. It was used to explore the creation and management of the proposed platform dedicated to improving the diagnosis and treatment of rare diseases.

The first section focuses on the two key user groups: participants, who include patients and healthy volunteers, and investigators, who include researchers and institutions. It aims to identify and analyse each user's profile and gain insight into their behaviours, expectations, and challenges. This analysis will also show how each user affects others. It was conducted by comprehensively reviewing existing academic literature, reports, and case studies. The first section will form the basis for understanding how the platform can best meet their needs.

The second and third sections of the thesis focus on developing strategies for platform design, with a particular emphasis on value creation and value capture. A theoretical framework for comprehending the dynamics of two-sided platforms is used. More academic sources were incorporated to enhance this framework, providing different viewpoints and guaranteeing a comprehensive examination.

Overall, this research-based methodology combines theoretical insights with practical considerations to develop a business plan that aligns with the platform’s mission of advancing rare disease diagnosis and treatment. By leveraging a mix of essential texts and supplementary sources, the thesis aims to provide a comprehensive guide to designing and managing a sustainable and impactful platform.

1. Uncover Value

This chapter examines the needs and expectations of participants and investigators to determine the best strategies for collaborating to mutual advantage.

1.1. Understanding Participants

To ensure clarity in this thesis, let us define a participant and who it may describe. This section will explore their profile, jobs, pains, and gains. We will also define what jobs, pains, and gains stand for in « 1.1.2 Participants Jobs, Pains, and Gains.»

Definition: A participant is an individual who voluntarily contributes data, biological samples, or experiences to research studies aimed at improving the diagnosis, treatment, and management of health conditions, including rare diseases. Participants may include patients affected by the condition studied, as well as unaffected people, family members, and caregivers.

1.1.1. Participants Profile

A rare disease also called an orphan disease[1], is any disease that affects at most five people out of 10,000 people. However, due to the number of rare diseases existing, between 6000 and 8000 different rare conditions have been identified to date (Radu et al., 2021); we count more than 350 million people worldwide affected by these (Sequeira, Almeida et al., 2021). Another article states that in the European Union, it is estimated that 30 million people are affected (Radu et al., 2021).

Most rare diseases, approximately 80% of them, have a genetic origin. They likely have a wide variety of symptoms and signs that vary, not just from one disease to another but also from one patient to another with the same disease. This common trait of relatively common symptoms through rare diseases can lead to misdiagnosis. It is a real issue that patients all over the world are facing; it is estimated that around 40% of patients receive an incorrect diagnosis during the time of their diagnosis. That time can take from 5 years to 30 years for 25% of patients if they ever get one diagnosis (Radu et al., 2021). During that moment,

[1]In some countries, a distinction is made between a rare disease and an orphan disease. "Rare disease" would refer to a condition with low prevalence; "orphan disease" would emphasise the lack of investment in research and treatment due to limited commercial interest. (*Rare And Orphan Diseases*, 2024)

patients and their families often receive several possible diagnoses before a definitive one is reached, resulting in delayed and/or missed opportunities for treatment, prevention, or even counselling (Laurie et al., 2022).

Another challenge met in the context of rare diseases is the geographic dispersion of patients. Patients affected by a specific condition are often scattered across vast geographic areas or in different institutions, which makes it difficult to compare and access reliable information on the natural history of each. This issue is made worse because many countries lack required patient registries, making it nearly impossible to determine the prevalence and epidemiology of rare diseases within populations (Radu et al., 2021). It is well known that patient comparisons can significantly help find new solutions and speed up the diagnostic process, especially for rare diseases. However, the limited number of potential participants makes each one extremely valuable in this context. Geographic dispersion exacerbates the difficulty in the process of recruitment of participants for clinical trials and research studies. To resolve this issue, national, inter-institutional, and international collaborations are essential (Javaid et al., 2016).

As said earlier in the definition, participants can be patients affected by a rare disease. They are confronted with the issues that rare diseases are facing and have most certainly been in front of a long process for diagnosis, no treatment, or lack of knowledge of their conditions because of the low research and low findings due to the dispersion of people with similar conditions. Participants are not only the patients themselves but also unaffected people, family members, partners, and caregivers who often play a critical role in care and support; they might also have faced those issues. It is, unfortunately, very common in this context.

1.1.2. Participants' Jobs, Pains and Gains

To better understand participants and their dynamics, it is important to look at their *jobs*, *pains,* and *gains*. Let us first define those key concepts. Jobs refer to the tasks participants try to perform, the problems they aim to solve, or the needs they seek to satisfy. There are three main types of jobs: functional jobs, social jobs, and emotional/ personal jobs. A combination of these is also possible. Pains denote the challenges and obstacles those

participants will face while performing their jobs. Finally, gains describe the desired outcomes and benefits participants would hope to achieve (Osterwalder & Pigneur, 2014).

1.1.2.1. Participants Jobs

Participants are primarily motivated by the hope that their contribution will advance medical research, particularly in the development of diagnoses and treatment for rare diseases. Whether they are a patient affected by a rare disease or unaffected people, sharing their health data is seen as a means to contribute to solving a more significant problem. Their health information will be helpful in understanding and completing research. The more information is available, the better chance there is to foster the development of diagnosis and treatment methods for rare diseases. Their participation, consisting of making their information available and sharing data, involves joining studies, providing biological samples, and continuously updating their health information through different surveys and other data collection methods, which is driven by this hope. The more comprehensive the data, the better the chances of advancing research. In some cases, however, the present information is not sufficient; the entire medical history of a patient may be required to fully understand the natural progression of rare diseases (Javaid et al., 2016).

A key element for enhancing data collection and their knowledge and awareness concerning the specific research would be for them to be actively involved in the research process. Being active can simply be participating proactively in activities suggested by the research or being active on the platform or app used. This involvement informs them about the progress and outcomes (Fürstenau et al., 2021).

1.1.2.2. Participants Pains

The two main pains a participant can encounter when allowing data collection are psychological and monetary costs. Indeed, these are important in the context of rare diseases, as the psychological burden and costs related to medical care can be significant. Therefore, it is important to consider them when addressing a platform for participants related closely or not so much to rare diseases.

Psychological costs are categorised as stress, anxiety, frustration, fear, and emotional fatigue. Indeed, they will face the fear of losing control over their data when transferring them. They will be concerned about how their data will be used, stored, and protected. Participants are susceptible to the extent of their consent to data privacy regulations. It is important to inform participants that data sharing is based on the principle of transparency and fairness, that they have the power over their consent, and that they are willing to share as much data as they want. Importantly, it states that participants' data must not be used as advertising to gain revenue, as it would risk losing their consent (Fürstenau et al., 2021).

Monetary costs can be significant in the context of medical care. For the patients and families, it can become a real problem to deal with treatment costs, costs of hospital stay, and other involved costs, which might include the costs of travel if needed, the costs of medical tests, and the potential loss of income due to sick leaves or stop working.

1.1.2.3. Participants Gains

The more important gains are not monetary. Indeed, participants want to be genuinely involved in the research process. They hope this will speed up the discovery of better diagnosis and treatment methods and make a real difference. If their involvement can make a difference in their life or that of someone else affected by a rare disease, it will matter.

They also want to have control over their decisions to respect their wishes and health needs. To do so is also to have knowledge about what is possible and done in some other places. Participating in a research project can help them in that matter, as they will not only feel involved in a task that is dear to them, but they will also learn more about their conditions and the possibilities open to them.

One last benefit present for participants is the community and the network they will be able to create and have while participating in such research projects. They will be able to connect with patients and families facing the same difficulties, allowing them to feel better understood and as a part of something bigger, a community.

To conclude, a compelling value proposition that could be proposed to participants should address key challenges, such as psychological burdens, data privacy concerns, and

monetary costs, while still empowering participants with control over their data and consent. By tackling those challenges associated with data collection and ensuring transparency in data usage, the platform can improve and reassure participants of their engagement with the project. This strategy can result in more positive research outcomes and benefits for both patients and researchers.

1.2. Understanding Investigators

To maintain clarity in this thesis, we will define what an investigator means and who falls under this category. This section will explore their profile, jobs, pains, and gains.

Definition: An Investigator refers to an individual or organisation who is involved in the research study of conditions, including rare diseases. They aim to conduct scientific investigations to advance knowledge and comprehension in certain conditions/ rare diseases. Investigators may be medical professionals, researchers, or teams of researchers (in universities, public research institutions, or private companies).

1.2.1. Investigators Profile

The lack of data in the study of rare diseases presents difficulties for medical researchers. This knowledge gap affects the recommended standard of care and may prevent an accurate and timely diagnosis. These issues lead to new projects that seek to gather the available information on these rare diseases onto a single, centralised platform (Sequeira, Almeida, et al., 2021).

The investigator side of the platform can also be seen as the medical side. Indeed, investigators can be researchers, scientists, and doctors from universities, public research institutions, or private companies. Their primary objective is to improve the diagnosis and treatment of rare diseases by accessing omics data from both affected and unaffected people.

1.2.2. Investigators'' Jobs, Pain and Gains

Investigators can have different jobs depending on which profile they have; they can aim to do fundamental and applied research to understand rare diseases better, they can aim to

develop new drugs and therapies, or they can aim to integrate research findings into patient care to improve patient outcomes. Moreover, the central common point is that they will use the data to perform their desired research and help patients. Some concrete examples include using the platform/ software to help diagnose, taking notes on specific symptoms and medical data, suggesting diagnosis and treatment to colleagues to spread awareness, and sharing their experiences with other physicians (Jankovic et al., 2011).

Investigators also manage participant consent and ensure data privacy. They must adhere to ethical guidelines when accessing private patient data, which is essential, as participants pay close attention to this detail. One last job we can mention for investigators is publishing their research, sharing their funding with the world through reputable journals, and securing funding to support their studies.

Investigators will also face several significant challenges that will obstruct their progress. One first issue is the lack of patient participation in research, which leads to the lack of donations of biological materials or data. Investigators will also face a significant fragmentation of patients' data; it is often dispersed across different databases, registries, and/or biobanks. Protecting patient identity in de-identified databases, registries, or biobanks is another significant challenge, especially for patients suffering from ultra-rare diseases. In addition to that, there is often a lack of willingness for collaboration between participating researchers and organisations to share data (Thompson et al., 2014).

Lastly, data privacy laws and managing consent processes are complex. The administrative load can become time-consuming and costly.

All the pain points mentioned above highlight the difficulties investigators face during research. The limited available data makes it difficult to compile a comprehensive dataset for analysis. Investigators must address these challenges to improve the efficiency and effectiveness of rare disease research.

Investigators will also benefit from using a platform. One benefit is access to comprehensive data to enable more accurate and in-depth research. This will allow them to better understand and develop treatments for rare diseases. They will also gain more time to research as they will not have to manage participant consent, data storage, and

administrative burdens. A platform will also promote collaboration among researchers, doctors, and organisations, which can accelerate the normal pace of discovery and innovation.

With those benefits, there are more chances for successful research, which can lead to high-impact publications, increased funding opportunities, and the development of new diagnoses and treatments. These will ultimately benefit patients and advance the field of rare diseases, but they will also valorise the investigator's work.

To conclude, a compelling value proposition for investigators should tackle significant obstacles such as data fragmentation, lack of patient participation, and data-sharing reluctance while enabling investigators to achieve access to comprehensive data, reduced administrative burdens, and enhanced collaboration opportunities. The platform can enhance and reassure investigators' participation in the project by helping them move forward in fundamental and applied research, developing new drugs and therapies, and integrating research findings into patient care by decreasing related difficulties. This approach may lead to more fruitful research endeavours and advantages for investigators and participants.

1.3. Network Effects

Network effects occur when the value of a product or service increases as more people use it. The value of such a product or service lies in its ability to let economic agents interact with one another, and thus, it depends on the decisions of the other agents. Network effects are highly present and important in platforms as they are what make agents come in the first place. There are two types of network effects: cross-side and same-side network effects (Belleflamme & Neysen, 2023).

The first effect, the cross-side network effect, is when, in our case, participants have an impact on investigators and inversely. For example, the more participants on the platform who consent to the use of their data, the greater the statistical power and research potential for the investigators. Another example is that the more investigators present and focus on one rare disease, the more likely they are to find new methods to diagnose and

treat patients, which will benefit participants. What we can wonder now is, if there is one focus on one particular disease, will this impact the other diseases? If yes, is it positive or negative? The presence of research spillovers across different diseases is worth investigating. Indeed, focusing on one rare disease would be positive for one segment of participants but negative for the other as less time and effort is spent on their specific disease. Research spillovers can look like an overall negative effect, but this is not necessarily true. Indeed, some diseases share the same traits, so advancing one may as well advance another. Or, more straightforward, the attention drawn to the platform by the researchers could also benefit other diseases. Hence, the view on research spillover drastically differs from a more short-term to a more long-term view. The effects mentioned can be named positive network effects as they have positive outcomes. Negative network effects also exist and are usually the opposite of positive network effects.

The second effect, the same-side network effect, works differently as it is when one side will affect itself. For example, participants will positively affect one another by attracting others to the platform via word-of-mouth and decreasing any mental obstacles that would prevent them from participating. Another example can be that the more investigators join the platform, the greater the chance there is to take advantage of synergies between different specialities and competencies, which is advantageous to all of them. We can also point out a negative same-side network effect for investigators: they might compete with one another when publishing results in academic journals or securing research funding.

The two previous effects combined create a feedback loop; in this situation, we can qualify it as a positive feedback loop. It is a self-reinforcing mechanism that amplifies the original stimulus. More investigators draw more participants, and so on, as more participants draw more investigators. This means that the platform's value grows naturally once it reaches a specific critical size. The specific size is still hard to determine precisely, but in terms of timeline, we could expect to achieve that after 2 to 3 years; this will be discussed in the section “3.4.3. Profitability”.

Another way we can categorise network effects is to see if they are global or local. When a user is added to one location, global network effects will lead to an impact on users

everywhere. In contrast, local network effects are limited to a specific location, so an additional user impacts only those who are nearby (Belleflamme & Neysen, 2023). In the case of a digital platform linking participants and investigators in the study of rare diseases, global network effects are in order as a new user, investigator, or participant will affect research on a specific disease, regardless of its location. The global aspect of those network effects is what makes this platform even more necessary.

Moreover, external effects on a platform can happen, and they refer to the platform's effect on stakeholders and non-users, who could indirectly impact the business (Belleflamme & Neysen, 2023). They can be positive or negative external effects, but in this case, positive effects are predominant as they can advance medical research on many different diseases and in various places, which can encourage authorities, organisations, and foundations to support and encourage the platform.

2. Create Value

This chapter aims to define the platform's mission statement, develop a compelling value proposition, and assess its competitive position.

2.1. Mission Statement

The platform's primary goal is to create strong connections between research participants and investigators, fostering collaboration that enhances the sharing of valuable omics data. Ensuring transparency and ethical data management is key to building trust between these groups. It guarantees that data is handled responsibly while respecting participants' privacy and informed consent.

Adherence to high ethical standards in data gathering and research execution is equally important. These measures are crucial for ensuring data protection, maintaining participant autonomy, and encouraging the responsible use of health information. By emphasising these principles, the platform seeks to strengthen the research environment while upholding participants’ rights and cultivating trust and mutual cooperation.

Additionally, the platform aims to highlight participants' significant role in advancing scientific research and breaking down barriers to their involvement in research studies.

Collectively, these goals form the following mission statement.

Our mission is to connect participants and investigators in a way that promotes transparency, data protection, and ethical practices, creating a research environment that respects participant autonomy while advancing scientific discovery.

2.2. Multisided Value Creation

A tailored value proposition aims to pinpoint for each group of participants and investigators the tools and services that address their most pressing jobs, pain relievers that alleviate their primary pain creators, and gain creators that enhance their core benefits (Osterwalder & Pigneur, 2014). This approach is grounded in a thorough understanding of

both parties' needs, allowing for the development of a customised value proposition that effectively meets each stakeholder's specific requirements.

Figure 1 demonstrates a canvas example for investigators, visually representing the links between gain creators and gains, pain relievers and pains, and services and jobs.

Figure 1: Value proposition

It might seem logical to create separate canvases for participants and investigators, each tailored to their specific needs. However, this approach would miss the important aspect of value co-creation that occurs between users on the platform. The benefits users receive are not solely derived from the platform's features but, crucially, from their interactions with other group members. Additionally, designing separate canvases could lead to conflicting outcomes, as the expectations of participants and investigators may not always align.

To address this, it is important that the value proposition reflects the interconnected nature of these user groups. This calls for a different tool—the multisided value proposition. This tool is designed to determine which tasks the platform should handle and which ones should be managed by the other group to address the jobs, pains, and gains of a specific group (Belleflamme & Neysen, 2023)

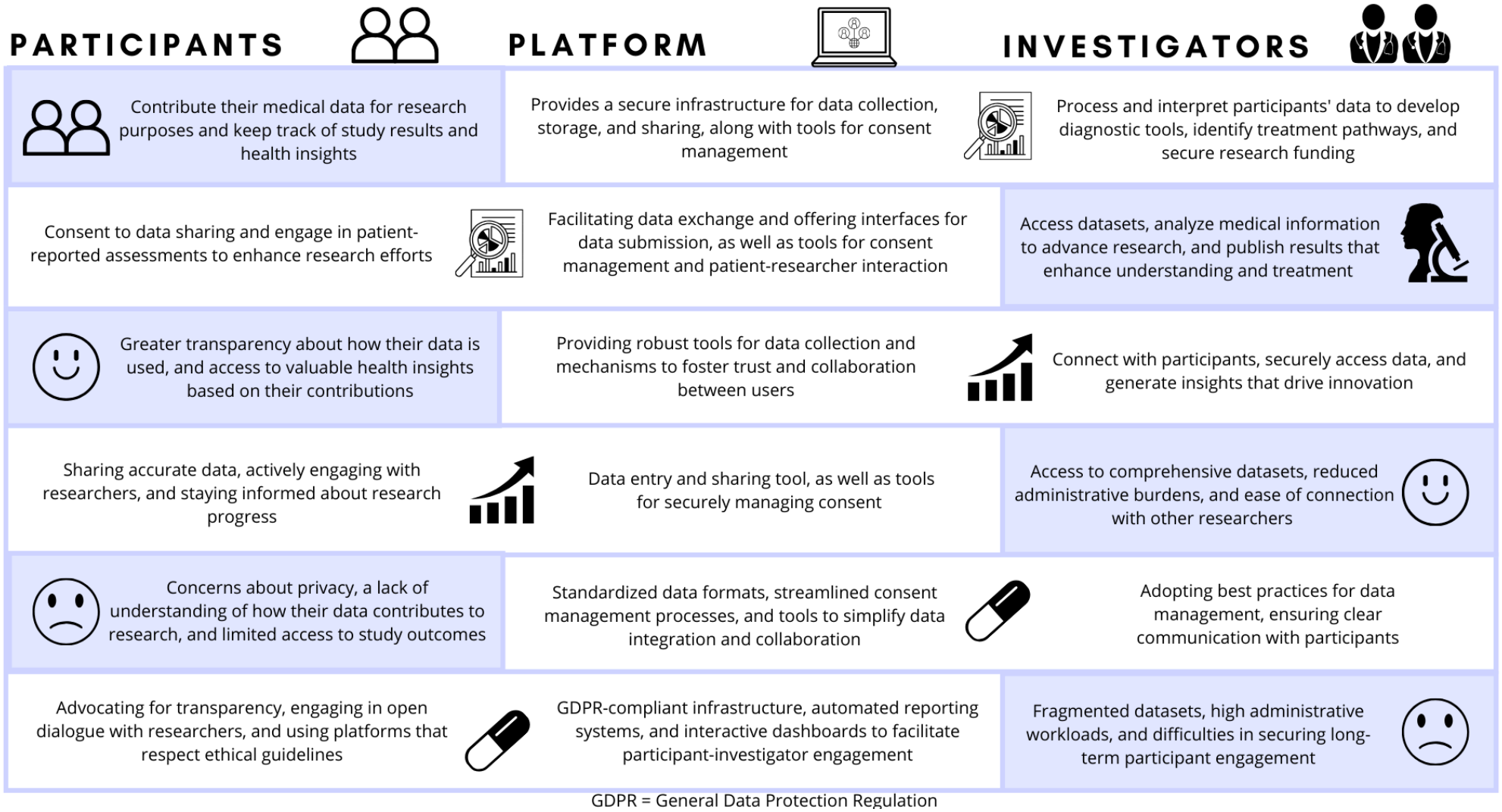

Figure 2: Multisided Value Proposition

Figure 2 illustrates a multisided value proposition for the digital platform that connects participants and investigators. The left column represents participants, the middle column focuses on the platform, and the right column pertains to investigators. Each row outlines the jobs, pains, or gains specific to each group, showing how the platform and the corresponding group address these aspects.

This figure demonstrates how a digital platform can effectively link participants with investigators. For example, the first row shows participant jobs (left), platform services (middle), and investigator services (right) that address these tasks. The second row reverses this direction, displaying investigator jobs (right), platform services (middle), and participant services (left). This structure is repeated for gains and gains creators (rows 3 and 4) and pains and pain relievers (rows 5 and 6). In order to understand the distribution of the value co-creation between participants and investigators, the multisided value proposition needs to be read horizontally. In order to identify potential complementarities or conflicts between the two groups, the structure must be read vertically.

The multisided value proposition emphasises the interdependence of the jobs, gains, and pains faced by both participants and investigators. These components are intricately connected, as addressing the needs of one group often enhances the experience of the other. For instance, when the platform supports an investigator's job of gathering comprehensive health data, it also helps participants feel that their contributions are advancing research. Similarly, alleviating a participant's difficulty in understanding how their data contributes to research benefits investigators by improving dataset quality and retention rates.

Figure 2 suggests that the platform should provide investigators with customisable research dashboards, allowing them to display their studies, data collection techniques, and findings. On the other hand, participants should have access to an intuitive interface to explore different studies, as well as detailed information on data usage and research updates. This interface should offer essential details such as study objectives, privacy

protections, compensation information, and participant responsibilities so that participants are well informed.

Furthermore, the platform should integrate tools for managing and interpreting participants' implications, as well as helping investigators recruit and maintain their study cohort. These tools allow investigators to communicate effectively with participants, create a sense of community, and highlight the impact of their study. This approach helps investigators differentiate their studies, ensure trust, and reinforce participants' implications.

2.3. Competitive Landscape

Figure 3 visually compares two well-known analysis models, Porter's Five Forces and the Value Net model, highlighting their main features and differences. Shared elements are shown in pink, elements unique to Porter's model are in blue, and those specific to the Value Net model are in purple.

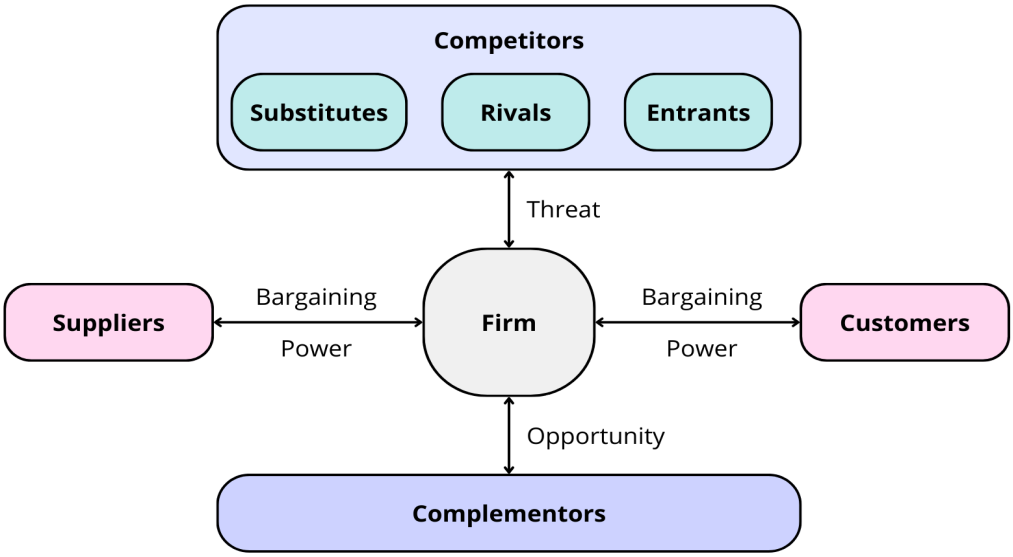

Figure 3: Porter's Five Forces and Value Net Models

Porter's Five Forces model, developed by Michael Porter in 1979, analyses an organisation's competitive landscape using five forces: (1) rivalry among existing competitors, assessing market competition intensity; (2) bargaining power of suppliers, evaluating their influence

on pricing and terms; (3) bargaining power of customers, gauging their ability to impact prices and demand; (4) threat of new entrants, measuring the ease with which new players can enter the market; and (5) threat of substitute products or services, examining the risk of customers switching to alternatives.

The Value Net model, introduced by Brandenburger and Nalebuff in 1996, extends Porter's framework by incorporating the concept of coopetition, a combination of competition and cooperation. This model introduces a new player category called complementors, which are entities that add value to a product or service when used together. The Value Net model groups rivals, substitutes, and potential new entrants from Porter's model into a single category labelled competitors.

Traditional models like Porter's Five Forces and the Value Net model are often inadequate for analysing digital platforms involving participants and investigators, as they do not fully capture the distinct dynamics of these platforms (Belleflamme & Neysen, 2023). These models struggle with settings where participants and investigators are deeply interconnected, with network effects and mutual dependencies playing a crucial role. They also overlook the significant influence of participants, whose disengagement could create negative feedback loops that impact research outcomes. Furthermore, these models focus primarily on direct competition, ignoring indirect strategies such as leveraging data insights for research enhancement.

To address these gaps, the Platform Value Net model is suggested as a more suitable alternative. This model adapts the traditional Value Net framework to reflect better the unique roles and interactions between participants and investigators within the platform ecosystem (Belleflamme & Neysen, 2023). It replaces the conventional distinction between customers and suppliers with different user groups the platform connects, and it categorises competitors and complementors based on their effect on engaging participants and investigators. Competitors are those that hinder the platform's ability to attract or retain participants or investigators, while complementors help facilitate these connections.

Figure 4 depicts the Platform Value Net for a digital platform connecting participants and investigators, with competitors shown in pink and complementors in green. The

competitive environment for such platforms involves several key elements, including substitutes, rivals, new entrants, and complementors (Belleflamme & Neysen, 2023). Substitutes serve one user group without facilitating interaction with the other, while rivals connect both user groups, making it more challenging to attract or retain users. Rivals can be direct, using a similar approach to the platform, or indirect, employing different methods. New entrants include potential future competitors and substitutes, while complementors enhance the platform's value by helping attract or retain users.

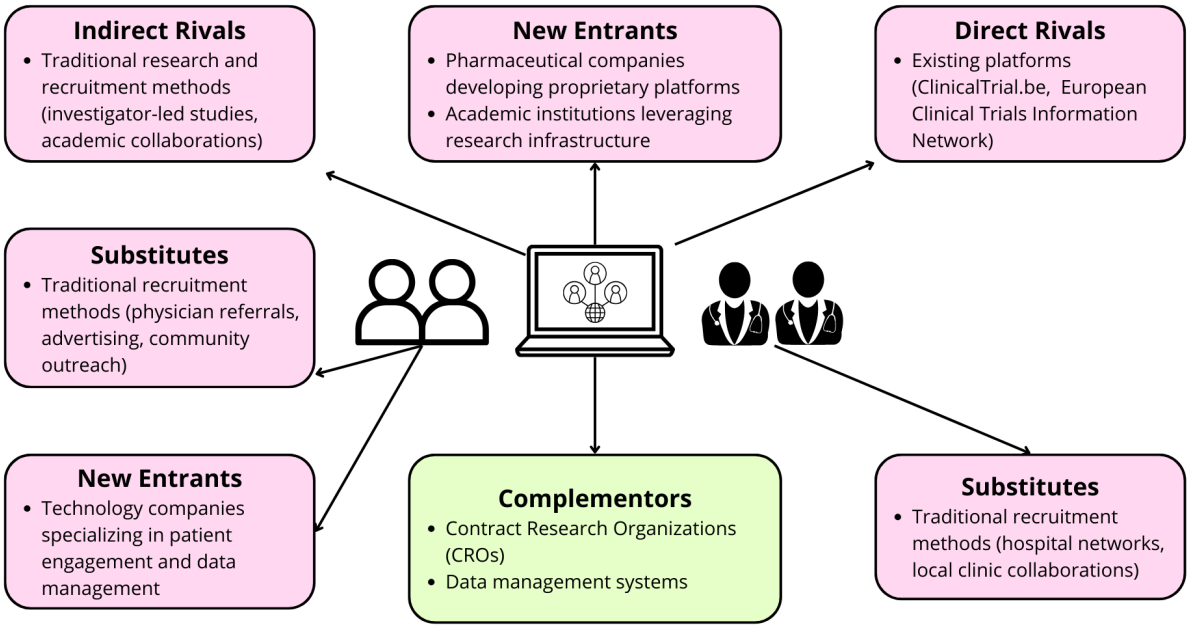

Figure 4: Platform Value Net

For participants, substitutes include traditional methods of finding clinical trials, such as physician referrals, advertising, or local community outreach programs. These methods are often limited in scope and may not provide participants with a comprehensive view of available trials. Potential new entrants could be technology companies specialising in patient engagement, leveraging advanced algorithms and data management systems to simplify the trial-matching process and enhance accessibility. They would offer alternative ways for participants to share data (*Clinical Trials In Human Medicines | European Medicines Agency (EMA)*, s. d.).

For investigators, substitutes include traditional recruitment methods such as direct outreach to patients through hospital networks or collaborations with local clinics. These approaches can be time-consuming and may lack the efficiency and scope of a digital platform. Complementors for investigators include Contract Research Organizations[2] (CROs), which assist with various trial-related activities such as protocol development, site management, and patient recruitment. Additionally, data management systems like the Clinical Data Interchange Standards Consortium (CDISC) Open Rules Consultancy play a crucial role in clinical research. CDISC, a global standards organisation, establishes frameworks to streamline clinical trial data management and ensure compliance with regulatory requirements. Its Open Rules project leverages open-source software to implement executable data conformance rules, enabling efficient data validation, quality assurance, and regulatory submissions. These tools enhance the overall efficiency of clinical trial processes by improving data accuracy and ensuring alignment with industry standards (*CDISC & CDISC Open Rules Consultancy*, s. d.) (Caruso, 2020).

The platform faces indirect competition from conventional research and recruitment methods, including investigator-led studies and academic collaborations without a dedicated platform. This indirect competition can lead to positive reinforcement; research performed outside the platform could nurture research conducted on the platform, and the other way around is also possible. Direct competitors include existing platforms like ClinicalTrial.be and the European Clinical Trials Information Network, which facilitates connections between patients and trial centres, particularly in niche areas like rare and serious diseases. (Caruso, 2020)(*60,000 Fewer Clinical Trial Places For Europeans, Despite Global Surge In Research Projects.*, s. d.). Potential new entrants include pharmaceutical companies aiming to develop their own platforms for trial recruitment and academic institutions leveraging their research infrastructure. For instance, AstraZeneca's Evinova[3]

[2] "Contract Research Organizations (CROs) are companies that provide research services to pharmaceutical, biotech, and medical device companies, as well as government agencies, foundations, and academic institutions.CROs offer a variety of services, including preclinical research, clinical trial management, data management, biostatistics, and regulatory affairs (issued by regulatory authorities like the FDA)." (Park & Park, 2023)

[3] Evinova, a health-tech initiative launched by AstraZeneca, offers globally-scaled digital solutions to optimize clinical trial design and delivery. Drawing on technologies used in AstraZeneca's trials across 40+ countries, it aims to reduce the cost and time of drug development while enhancing patient experiences through remote monitoring and digital therapeutics. Evinova also collaborates with partners like Parexel and Fortrea to

initiative exemplifies how pharmaceutical companies integrate globally scaled digital health solutions into clinical trial design and delivery systems. By leveraging technologies from trials conducted in over 40 countries, Evinova not only enhances trial efficiency and reduces costs but also prioritises patient experiences through innovations like remote monitoring and digital therapeutics.

Complementors are essential to the platform's value proposition, offering services that enhance data collection and research insight while fostering trust. For example, CROs can provide critical support in streamlining trial processes, from recruitment to compliance management.

The analysis highlights a highly competitive landscape where each group has multiple alternatives. To stand out, the platform should provide investigators with user-friendly research tools that increase study visibility, simplify participant communication, and optimise data collection processes. Collaborating with leading CROs could offer investigators enhanced capabilities, including access to advanced research tools and expertise. (Caruso, 2020)

The platform should prioritise usability for participants by simplifying the process of finding and enrolling in trials. Existing platforms often offer limited features due to budget constraints or narrow research focuses. This platform can differentiate itself with a robust interface, advanced search functionality, and personalised study recommendations. Additionally, ensuring a wide range of available studies without restricting the scope to specific diseases or demographics will help participants avoid navigating multiple platforms.

To help visualise the competitive landscape and where the platform stands compared to its competitors, we can look at Figure 5, the competitive matrix. The two chosen dimensions are relevant for comparing the platform with its competition in this sector. The vertical axis represents "Technological differentiation"; it emphasises the technological advancements and unique features that can differentiate the competitors, highlighting the level of

provide comprehensive support for stakeholders such as clinical trial site teams, CROs, and patients, underlining its role in driving digital transformation in the life sciences sector. For more information, see AstraZeneca's press release.

innovation and each solution's capabilities. The horizontal axis represents the " Scope of impact", which emphasises the extent of the reach, both geographically and in terms of the range of rare diseases it covers. Thanks to that matrix, we can see where each solution is and what its competitive advantage is. The platform is placed as a high technological differentiation and scope of impact, notably due to the differents features it must have to stand out, explained earlier in this chapter. Indeed, more traditional recruitment methods are less technologically differentiated, for example. In reality, the platform would start a bit lower on the scope of impact, as it will be restricted to one region, but as time goes on, it will expand, so it is already placed in the upper right corner. Lastly, the platform is comparable to other platforms already discussed in the chapter, which is an additional reason to have added value in features to make sure to stand out.

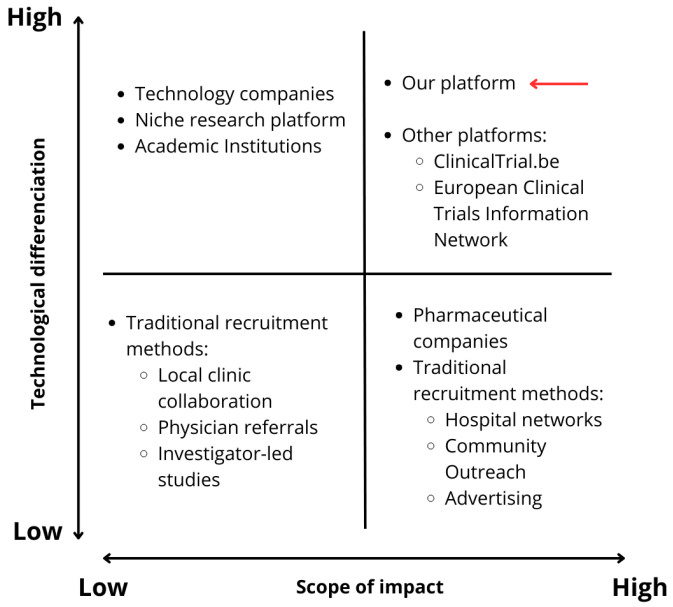

Figure 5: Competitive Matrix

2.4. Defensibility strategies

When a platform connecting participants and investigators establishes a competitive advantage in the research field, the key challenge becomes maintaining that advantage over time. This capacity, known as defensibility, hinges on two main factors: specificity and

replicability (Belleflamme & Neysen, 2023). Specificity refers to how exclusive a platform's benefits are to its users. High specificity means the platform's unique advantages are accessible only to its users, while low specificity means that similar benefits are available across multiple platforms.

To reduce specificity, platforms can allow for cross-platform interactions or lower the costs associated with users participating on multiple platforms simultaneously (multi-homing). This approach maintains interactions within each platform, but the low cost of multi-homing allows users to reap the benefits of several platforms concurrently.

A high-specificity environment often creates a winner-takes-all situation, where platforms fiercely compete for dominance, with one emerging victorious and others failing. However, this scenario can be volatile and uncertain. As a result, it may be more strategic for a platform to strike a balance in specificity, enabling healthy competition and the coexistence of multiple platforms in the market. (Belleflamme & Neysen, 2023)

For a platform that connects participants and investigators, specificity is decreased by insufficient data quality, simplistic matching algorithms, incomplete trial information, participant misrepresentation, and a lack of feedback mechanisms to refine matches. Future integrations with centralised clinical trial databases or clinical management tools[4] could further reduce exclusivity by making data management more seamless across different sources. On the other hand, forming exclusive partnerships with leading Contract Research Organizations can enhance the platform's uniqueness and create entry barriers for new competitors.

Replicability refers to the ease with which other platforms can imitate the successful outcomes of a platform. It is shaped by the efficiency of user matching and the coordination of users' decisions. Efficient matching between participants and investigators strengthens network effects, making them harder for competitors to replicate. When effective matching relies on having a large and diverse user base present simultaneously, smaller platforms often struggle to match the quality of service. Furthermore, users' ability to coordinate their decisions affects replicability; when coordination is complex, network effects create

[4] Such as the Shared Investigator Platform which supports a new approach for streamlining clinical trials.

collective switching costs. These costs discourage users from switching platforms, as they fear losing network benefits if not enough others switch with them. (Belleflamme & Neysen, 2023)

In a platform connecting participants and investigators, simultaneous engagement of both groups is not always necessary. A platform can initially focus on building a robust presence for one side, such as investigators listing trials, while gradually attracting participants. To enhance defensibility and create collective switching costs, the platform should offer a wide range of studies and include diverse investigators, avoiding limitations to specific research areas or participant demographics.

This strategy not only broadens the platform's appeal and reduces its replicability by fostering strong network effects that are difficult for competitors to imitate. Additionally, it addresses a common drawback of existing platforms, which often narrow their focus to specific fields or target audiences. However, while expanding the scope to lower exclusivity, it is crucial to maintain efficient matching systems and robust data quality. Balancing scale and specificity ensures that the platform remains competitive, defensible, and indispensable for both participants and investigators in the long term.

2.5. Takeaways

This section will highlight the different concepts in the chapter and summarise the main points. The chapter's main points for the platform are its mission, compelling value proposition, competitive position, and strategies for maintaining a long-term market advantage.

The platform's mission is to connect investigators and participants to promote a collaborative research community. Its goal is to prioritise ethical data sharing, transparency in data utilisation, and participant autonomy. This strategy aims to advance scientific discovery in the scope of diagnosis and treatment of rare diseases.

The multisided value proposition is suited to address the interconnected needs of both groups. Investigators need data handling, enhanced visibility, and streamlined recruitment. To fulfil those needs and improve the investigators' experience, customisable profiles,

research dashboards, and participant engagement tools must be used. Participants need transparency and reassurance; it can be achieved with a user-friendly study directory with detailed information on the study objectives, how their data are used, and the protection of their privacy. This will allow them to make informed decisions and encourage them to participate actively. All those discussed features will help researchers present their research effectively and enable participants to make informed decisions about their involvement. This will help bring valuable data to the research ecosystem.

The competitive landscape analysis outlined the need for differentiators so that the platform could stand out in this market. To achieve this, specific tools must be present for each group. For investigators, easy-to-use tools to facilitate participant recruitment are essential, as well as optimise data management tools and potent tools to grow their research visibility. For participants, an intuitive interface is key; it will simplify the search for relevant studies. Access to a broad range of study topics is also an added value; the aim is to avoid the limitations to specific demographics or research areas when the platform is well developed.

The defensibility strategy highlighted building alliances between the platform and complementors to enhance its value and credibility. In terms of complementors, they include contract research organisations and data analysis services, for example. In addition, collective switching costs should be created to reduce participant's need to rely on and switch to alternative platforms. To do so, the platform needs to include a wide array of investigators and research fields, ensuring that participants can access multiple studies without being limited.

3. Value Capture

This chapter explores the platform's value capture elements, focusing on its development, launch, and monetisation strategies. It then evaluates the project's overall profitability.

3.1. Platform development

To have an efficient release of the platform, a clear focus on delivering essential functionalities that create value for both participants and investigators is key. To accomplish this efficiently, the process will begin with creating a Minimum Viable Platform (MVP). This initial version will prioritise the fundamental features needed to test and validate the platform's core concept with key user groups.

The launch of two-sided platforms, however, is often challenged by the “chicken-and-egg” problem. To attract users from one group (e.g. participants), the platform must first demonstrate value by attracting users from the other group (e.g. investigators) and vice versa. A standard solution to this dilemma is to “start small,” initially constraining the platform’s operations to address the primary needs of a limited set of users with a focused set of features. By targeting a specific user base, the MVP can act as a proof of concept, showcasing its value, building trust, and gathering critical feedback to refine the platform. (Belleflamme & Neysen, 2023)

As mentioned, the MVP will serve as the foundation for connecting participants and investigators. For participants, it will include tools to securely upload and manage health data, supported by an intuitive interface designed to minimise the effort required to participate. Key features for participants in the MVP will include robust protocols to ensure patient confidentiality, including data encryption and compliance with national and international privacy regulations. It will also allow data anonymisation. Another key feature is the system's user-friendliness; a simple system allows participants to easily share their medical records, symptoms and diagnostic histories while maintaining strict privacy protections.

For investigators, it will provide access to aggregated data support to support their studies, along with tools to identify trends and potential breakthroughs. Key features for investigators in the MVP will include tools enabling them to easily filter and analyse data on specific criteria, such as demographics, symptomatology, or disease progression. Another feature that would benefit investigators is the ability to request specific data sets or collaborate with organisations, such as patient advocacy groups[5].

The development of the MVP will be carried out by a dedicated team, including a project manager to oversee the coordination, a user experience (UX) designer to ensure accessibility for non-technical users, and two software developers specialising in secure database management and interface design. This core team will collaborate closely with medical professionals, patient advocates, and data privacy experts to align the platform's design with its stakeholders' needs.

The development process will follow an agile methodology, which involves breaking it into phases and emphasising continuous collaboration and improvement through regular feedback cycles. Development will proceed over a certain period of time, and after each phase, a review session will be held with selected participants and investigators to assess progress and suggest improvements.

The MVP will initially focus on a limited geographic area, such as a specific country like Belgium. This pilot phase will enable testing under controlled conditions, allowing the team to address potential challenges, such as variations in medical record formats or regulatory requirements. Insights from this phase will inform the global rollout, ensuring the platform's scalability and relevance across diverse contexts.

Given the complexity of the medical and technological domains, the platform's development will involve strategic collaboration with external experts. These collaborators will include cybersecurity firms to safeguard patient data, healthcare providers to validate the

[5] "Patient advocacy groups are organisations that are set up to represent and support patients and their families living with a specific condition. They are one of the few outlets that can be turned to for credible information, understanding and support. Patient support groups advocate for their community by hosting family conferences, leading research projects, launching awareness campaigns and recommending treatment for approval to regulatory bodies." (Beacon for rare diseases, 2023)

platform's medical relevance, and advocacy groups to enhance participant engagement. Additionally, partnerships with existing rare disease registries or databases may be explored to integrate complementary data sources seamlessly.

Feedback gathered during this MVP phase will guide further iterations, ensuring that the platform evolves effectively to meet its users' needs. Once validated, the MVP will serve as the foundation for scaling the platform's features and operations to a broader audience, supporting its long-term growth and impact.

3.2. Platform launching

Launching a platform that bridges the gap between participants and investigators in the context of rare diseases involves addressing the inherent challenges of two-sided markets, particularly the "chicken-and-egg" problem already mentioned in section *3.1. "Platform Development"*. A carefully crafted launch strategy, focusing on both User Acquisition Cost (UAC) and Attraction Power (AP), is essential to overcome this hurdle and establish a strong user base.

3.2.1. User Acquisition Cost

User Acquisition Cost (UAC) refers to the expenses or effort incurred in attracting one new user to the platform. These costs vary depending on the user group targeted and can include prospecting, easing the adoption, and trust-building activities.(Belleflamme & Neysen, 2023)

Looking first at prospecting costs, investigators find these costs relatively low as many are already part of professional networks, academic institutions, or industry organisations that can be targeted through direct outreach and partnerships. A more personalised approach, such as attending conferences or hosting workshops, can efficiently attract researchers to the platform.

Conversely, prospecting for patients is more challenging due to their dispersed nature and the sensitive nature of the information required. Effective strategies may involve leveraging

patient advocacy groups and collaborations with healthcare providers. While these channels are broader, they tend to be less personalised, potentially increasing costs.

To make it easier for users to adopt the platform, we must address two barriers: pessimistic expectations and specific assets. Pessimistic expectations arise when potential users doubt the participation of others, undermining the perceived value of joining the platform. To overcome this, the platform must provide users with stand-alone benefits derived from consuming platform services independent of other users' participation. Specific assets refer to the unique investments users need to make to participate on the platform, such as acquiring knowledge or tools that have limited value outside the platform.

It generally involves familiarising investigators with the platform's functionalities, such as data filtering and analysis tools. Since these users are often tech-savvy, training requirements are minimal, keeping onboarding costs low.

However, participants may face higher onboarding barriers, including difficulty understanding how to securely share medical data. The platform must invest in explicit instructional materials, such as videos and FAQs, and provide customer support to simplify the process.

Building trust is crucial for both user groups as they must feel comfortable interacting on the platform. The problem resides in trusting the other users; in this specific case, facilitators may be the reputation of investigators or the organisation in which they work. Investigators also need assurance about the quality and completeness of the data, while participants require confidence in data security and ethical handling. This requires transparent policies, strong data encryption technologies, and compliance with international standards such as GDPR. These measures may increase upfront costs but are essential to establish trust.

Overall, while investigators have a lower UAC due to their existing professional networks and fewer onboarding challenges, participants' UAC is higher due to broader prospecting and trust-building requirements.

3.2.2. Attraction Power

Attraction Power (AP) refers to the strength of the cross-side network effects between participants and investigators, determining the ability of one group to attract members of the other group. To evaluate attraction power, we can examine the scarcity of users, as well as the exclusivity and capacity to interact outside the platform.

The investigator's population is naturally smaller than the patient population, making investigators a "scarce resource." Their presence on the platform significantly enhances its value for participants, indicating a higher likelihood of meaningful research and actionable insights.

The platform significantly benefits both investigators and participants by addressing the challenges they face in connecting with each other. For participants, it provides a direct channel to engage with investigators and contribute to medical advancements, which can otherwise be difficult to access. Similarly, investigators often face significant hurdles in sourcing enough quality datasets essential for their research. By bridging this gap, the platform creates a mutually beneficial environment where both groups find opportunities that are otherwise challenging to secure.

Alternatives for investigators include other databases or research collaborations, while traditional clinical trials or advocacy group platforms may suffice for participants. However, the platform’s emphasis on rare diseases, combined with its commitment to transparency and collaboration, positions it as a distinct and non-substitutable option for both groups.

3.2.3. Strategy Elaboration

The platform launch strategy will focus on a phased approach, prioritising one user group to create value for the other and establish early network effects. The strategy will also focus on having an initial target group, creating incentives for early adopters, organising collaborative events, having stakeholder-specific messaging and maintaining a positive feedback loop.

The first priority will be on participants, as their participation provides essential data that researchers need to engage and participate on the platform. To facilitate participant onboarding, collaboration is key; for instance, cooperation with advocacy groups, rare disease registries, and healthcare providers, can facilitate participant onboarding.

Early adopters will receive incentives to ease the onboarding process. For example, researchers could benefit from free access to premium tools during the platform's pilot phase.

Webinars, workshops, and conferences would also allow both groups to connect and share their experiences. These events would build awareness and foster trust and engagement.

Each group will have its own personalised communication strategy appropriately developed. The main messages for participants will focus on their empowerment, privacy assurance, and the platform's ethical commitments. For investigators, the focus will be on rich, quality data availability and the tools that will transform their way of working to make it easier.

Continuous feedback from early users plays a crucial role in shaping the platform’s development. Participants and investigators involved in the pilot phase provide valuable insights that guide iterative improvements, enhancing user satisfaction and retention. This early engagement not only refines the platform but also lays the groundwork for a key mechanism in platform dynamics: feedback loops.

Feedback loops were already explained in the section “1.3. Network effect” but as a reminder, there are self-reinforcing mechanisms that amplify the platform’s value over time if they are positive. A positive feedback loop occurs when participants' engagement increases the dataset's quality and richness, attracting more investigators. As the investigator's base grows, the platform becomes more appealing to participants, creating a cycle of organic growth that requires minimal additional effort to sustain. This dynamic is critical for the platform’s scalability and long-term success. The platform’s strategy will focus on creating and maintaining positive feedback loops to ensure sustained growth. This

involves fostering early engagement on both sides by providing stand-alone benefits, ensuring a high-quality user experience, and building trust.

The platform can establish the network effects necessary to drive sustained growth and long-term success by strategically prioritising patients in the initial launch and demonstrating clear value to investigators. The phased rollout approach, combined with tailored incentives, strong feedback loops, and targeted messaging, positions the platform as a transformative tool for advancing rare disease research.

3.3. Platform Monetisation

The platform's monetisation is a critical component of its sustainability and long-term success. The chosen strategies must align with the platform's mission to facilitate better diagnosis and treatment of rare diseases while ensuring accessibility for participants and value for investigators. The revenue model must also support the platform's ethical ambition to empower patients by maintaining free access for participants. This approach is not only ethically necessary but economically sensible, as free access encourages higher participation rates, strengthening the platform's network effects. To balance profitability and accessibility, the platform will employ a multi-faceted revenue model that integrates both primary and secondary income streams, ensuring financial viability without compromising its ethical commitments.

3.3.1. Revenue

3.3.1.2. Primary Revenue Streams

The primary revenue stream will be a subscription-based model targeting researchers, institutions, and pharmaceutical companies. These subscriptions will grant users differentiated access to the platform's advanced features and datasets. For instance, the basic level will provide limited access to aggregated, anonymised data for preliminary research. The premium level will enable advanced analytics tools, downloadable datasets, and the ability to request specific data subsets, for example, by region, demographic or any

specific conditions. It will also allow collaborative opportunities with patient advocacy groups.

This model ensures that the core functionalities remain affordable for individual researchers or smaller institutions while providing advanced capabilities for larger, well-funded organisations.

Given the platform's focus, another primary revenue stream option iss in the possibility of securing funding from organisations and initiatives dedicated to advancing medical research. Public grants from public health agencies or international initiatives like the European Union's Horizon funding programs could support initial platform development and specific projects. Additionally, sponsorships from pharmaceutical companies seeking patient insights or trial participants can serve as an additional stable revenue stream.

3.3.1.2. Secondary Revenue Streams

With participant consent and strict adherence to privacy regulations, the platform can enter into data utilisation agreements with research institutions or pharmaceutical companies. These agreements provide access to anonymised datasets for purposes such as identifying trends, informing drug development, or optimising clinical trial designs. Revenue from these agreements will be reinvested into the platform to expand its features and improve user experience.

The platform can also generate additional income by operating specialised services and solutions outsourced from the platform's infrastructure. This "food truck mode" involves providing analysis, consulting, and tailored services on demand, extending its reach while maximising operational efficiency.

The platform could also generate revenue by organising events and workshops, such as virtual conferences, patient-researcher matchmaking sessions, or educational webinars. Participation fees from attendees or sponsorships from healthcare companies could further diversify the platform's income streams while strengthening its community engagement.

Guaranteeing free or subsidised access for participants is a cornerstone of the platform's mission. This strategy recognises that participants play an essential role in the platform's network effects. The more participants there are, the more attractive the platform becomes to investigators, creating a virtuous circle of engagement.

The subsidy model is supported by revenues from public bodies, charitable funds and the diversified revenue streams described above. These sources enable the platform to maintain accessibility for participants while generating sufficient funds to support growth and innovation.

3.4. Platform profitability

3.4.1. Costs

The costs associated with the platform can be categorised into fixed costs and variable costs; fixed costs do not fluctuate according to the platform's activity, while variable costs do and are more related to the development of the platform's activities.

3.4.1.1. Fixed Costs

Fixed costs are those that remain relatively stable, regardless of the volume of users or platform activity. These costs are necessary to maintain the platform's core infrastructure and ensure compliance with legal and operational requirements.

Initial investments in creating the Minimum Viable Platform (MVP) will be present, including hiring employees like software developers, user experience designers, and cybersecurity specialists. These costs are substantial during the development phase but transition to lower maintenance costs after the MVP launch. Salaries for other core personnel, such as platform administrators, data scientists, and customer support teams, are necessary for daily operations and can also be counted as fixed costs.

Fixed fees for cloud services, software licenses, and IT infrastructure essential for platform operations will exist. While usage-related fees may vary, the baseline cost of maintaining a reliable infrastructure is relatively constant.

In the initial stages, marketing costs will remain fixed to ensure consistent efforts to establish a strong user base. These include costs in patient recruitment and in building community, such as partnerships with advocacy groups, online campaigns and educational initiatives, or even hosting webinars, workshops, and collaborative events. Other costs include targeted email marketing, participation in academic conferences, and leveraging professional networks.

3.4.1.1. Variable Costs

Variable costs fluctuate based on the platform's activity level, including the number of users and the scope of operations. These costs increase as the platform grows.

Cloud hosting costs will scale with the volume of patient and researcher data processed. As more users join, storage and computational requirements increase, driving up associated costs.

The cost of audits and system updates to maintain international privacy standards may also increase with the complexity of handling larger datasets. As well as regular investments in encryption technologies, legal consultations, and adherence to GDPR and other privacy standards ensure trustworthiness and compliance.

3.4.3. Profitability

To manage and attain profitability, a good equilibrium is needed between the initial investment and establishing a scalable revenue model. As we remain in a theoretical framework, it is hard to have exact projections, but the platform’s strategy was made to achieve financial sustainability within a reasonable timeframe. The following steps describe the planned progression to profitability:

For the first year, the focus will be on the platform's development, the recruitment of participants and the onboarding of investigators. During that first phase, revenues are expected to be minimal as effort will be spent building the user base and improving the platform.

For the second year, user adoption is anticipated to grow, supported by the introduction of paid subscriptions and data utilisation agreements. However, costs will probably be high because the platform is still developing to scale its infrastructure and conduct outreach efforts.

In the third year, a mature user base should be established, thus enabling the platform to generate revenues from regular subscriptions while diversifying income through secondary revenue streams. Investment in marketing may decrease due to organic growth and word-of-mouth, which are starting to take place.

The goal is for the platform to become profitable by leveraging economies of scale, a diversified revenue model and sustainable user engagement. However, the specific planning and financial outcomes depend on user adoption, operational efficiency, and the effectiveness of the monetisation strategy. Obtaining positive cash flow will be a key step thanks to iterative improvements and ongoing refinement of the platform’s business model.

Beyond profitability, it is important to highlight that the platform plans to align its financial success with its mission. The surplus revenue will be reinvested in initiatives that are centred around the platform’s community, such as supporting small-scale rare disease research projects, subsidising patient fees, and improving the platform's accessibility. These initiatives show the platform's engagement towards progress in the healthcare field for rare disease patients. The platform's fundamental mission to connect investigators and participants is to help find an appropriate treatment for rare diseases. It also favours collaboration within the medical community. This supplementary focus guarantees that the platform's durable objectives stay connected to its broader mission.

3.5. Takeaways

This chapter on Value Capture outlines a strategic framework to ensure the platform’s financial sustainability while advancing its mission to improve rare disease diagnosis and treatment.

The platform’s development focuses on a Minimum Viable Platform (MVP) with essential functionalities. It is guided by agile methodology and feedback loops to ensure user-centric

evolution. Partnerships with key stakeholders further enhance its technical, ethical, and community alignment.

To address the challenges of a two-sided platform, the launch strategy prioritises patient recruitment to establish a strong data foundation for attracting researchers. Fixed early-stage marketing efforts, with ethical positioning, trust-building, and streamlined onboarding, will reduce User Acquisition Costs (UAC) and maximise Attraction Power (AP). Positive feedback loops, driven by growing user engagement, will create organic growth and network effects.

The monetisation model balances inclusivity and sustainability, offering participants free access while generating revenues through subscriptions, grants, and secondary income streams such as anonymised data agreements and external partnerships. Achieving profitability depends on scaling through network effects and diversifying revenues, with surplus profits reinvested in community-focused initiatives like subsidising participant fees and funding rare disease research projects.

By balancing financial goals with its ethical mission, the platform provides a scalable model for transforming participants-investigators collaboration while contributing to broader healthcare innovation.

Conclusion

As a reminder, this master's thesis aims to answer the following research question: "How to improve the diagnosis and treatment of rare diseases?". Rare diseases affect millions worldwide yet remain underrepresented in healthcare research, leading to misdiagnosis, delays in treatment and, thus, untreated patients. To answer this question, this thesis proposes a two-sided digital platform to bridge the gap between patients and investigators. The aim is to accelerate diagnoses, improve research outcomes, and advance treatment through a platform connecting both user groups and enhancing data sharing and communication. This concluding chapter reflects on the summarised key findings, outlines limitations and identifies avenues for future research.

In the first chapter, "Uncover Value", we examined participants' and investigators' profiles and discussed their jobs, pains and gains, and the network effect between the two groups. The key findings are that participants prioritise data security, privacy and transparency, and user-friendly interfaces and that investigators require streamlined data access, robust analytics and seamless collaboration tools. It was also pointed out that network effects play a vital role in fostering the platform's growth and that trust needs to be present to promote collaboration. Both cross-side interactions and same-side interactions drive it. Cross-side interactions can be data sharing between patients and investigators, and same-side interactions can be discussions and sharing of findings from one group of investigators to another.

In the second chapter, "Create Value", we established the platform mission, which focuses on fostering collaboration while ensuring ethical data sharing and participant autonomy. To make the platform stand out, we also highlighted unique features, such as customisable research dashboards, advanced analytics, intuitive participant interfaces, and integration of disease-specific research tools; those features were identified as differentiators. They collectively ensure the platform addresses unmet needs in rare disease research and patient care. Strategies were mentioned to maintain its competitiveness, including forming

partnerships with patient advocacy groups and healthcare institutions. The goal would be to enhance the platform's credibility and reach.

In the third chapter, “Capture Value,” the practical execution of the platform’s objectives was focused. To create the platform successfully, a phased rollout strategy was recommended. It was suggested that we start with patient recruitment to build a fundamental dataset that would attract investigators. The number of participants needed to attract investigators depends. Indeed, 10 participants could already be something for some specific disease; for others, 1000 might be required. To maintain the platform's financial viability without sacrificing accessibility, the suggested monetisation models include donations from health organisations and tiers of subscription plans for investigators. The strategy also emphasised the importance of expanding the platform while complying with local regulations.

Limitation:

Although the work provides new and interesting insights, it is important to highlight certain limitations of our research and the shortcomings of our methodology.

The first limitation is the absence of empirical testing, which poses a challenge in validating the final strategies. Even if the conceptual framework gives a solid foundation to understand and start on strategy, the lack of real-world testing limits the ability to assess results. It is not easy to prove that the platform would effectively meet user needs and achieve the desired objectives. Conducting pilot studies with small, diverse user groups could address this limitation. It would offer practical insight into user behaviour, engagement, and platform functionality. Creating those studies requires extensive thinking and rules as it is in a medical environment, and it should not invade patients' privacy or play within the limits of the researcher’s medical responsibility of confidentiality.

The second limitation concerns the platform’s scalability. Although it was considered conceptually, the practical challenges of adapting the platform to different healthcare

systems and cultural contexts remain unexplored. This limitation highlights the need for future research into region-specific adaptations to guarantee the platform's global applicability.

Further Research:

After discussing this thesis's limitations, some recommendations for future research emerge. First, pilot studies are a first proposition to validate the proposed strategies and refine the platform’s functionalities. These studies should involve diverse user groups to ensure that the platform meets the needs of patients and investigators in varying contexts.

Second, it is interesting to investigate the platform's scalability further. Research should focus on how the platform can adapt to diverse healthcare systems, regulatory environments, and cultural contexts, looking into multiple regions and regulations. This includes identifying potential barriers to adoption in the different areas and developing localised strategies to overcome them.

Lastly, longitudinal studies examining the long-term effects of the platform use on rare disease research and patient outcomes would provide valuable insights into the strategy's effectiveness. Such studies could assess whether the platform achieves its intended goals of accelerating diagnoses, enhancing collaboration, and improving treatment options over time.

This thesis represents an initial step toward addressing the systemic challenges in rare disease diagnosis and treatment through digital innovation. Of course, multiple ways exist to improve the diagnosis and treatment of rare diseases. Here is one potential action to transform the rare disease landscape and suggestions for further research.

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Abstract:

Rare diseases affect between 350 and 475 million people globally, according to the estimation of the World Economic Forum. Approximately half of those people are children. Those numbers pose extraordinary challenges for patients, caregivers and the healthcare system. Although individually uncommon, these diseases collectively impact millions, yet more prevalent conditions often overshadow them in terms of research funding, public awareness, and policy focus. This causes severe difficulties for patients, such as delays and challenges in accessing adequate diagnosis, creating prolonged diagnosis journeys; they can also encounter insufficient access to specialised care, and as a result, this can cause high economic burdens on their families but also on society. This lack of attention to rare diseases causes a slowdown in research and innovation and thus creates a cycle of underdiagnosis and inadequate treatment.

This thesis aims to provide a comprehensive business plan for the proposed platform, it covers its development, launch, and long-term sustainability. This research presents actionable strategies for creating and capturing value, drawing from various academic and industry sources. The analysis is structured into three key sections: Uncover Value, Create Value, and Value Capture.

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