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Initiating and Defining a Sustainable Project on the Example of Rare Disease Therapy

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Abstract:

Purpose: *The main aim of the article was to present the possibilities of improving the definition of orphan therapy implementation projects using the P5 GPM model in the context of reimbursement decisions.*
Design/Methodology/Approach: *Theoretical research aimed at verifying the applicability of the P5 model in the practice of managing the implementation of orphan therapies. In the first part, the authors presented the essence of defining the project. Then, the background for the implementation of orphan therapies has been presented.*
Findings: *The article presents the long-term impact relating to the limitation of the development of innovation and cooperation, as well as competences of the medical staff and the medical environment, exclusion of the patient and his family from the labour market, the increase in logistics costs related to attempts to reach the treatment, which has an impact on the natural environment. All of this results in a reduction in national welfare. The authors indicated that the use of the P5 model allows for a comprehensive analysis of the environment of orphan therapy implementation projects both in the short and long term, linking them with the goals of sustainable development, which in turn will ensure the correct definition of the project and making investment decisions.*
Practical Implications: *Possibility to use the concept in the health technology assessment process for the purposes of the reimbursement decision, taking into account the sustainable development goals. The P5 model can be helpful in defining these types of projects.*
Originality/Value: *A look at the logic of intervention in case of reimbursement of rare disease therapies from the perspective of the SDGs. Indication of the broad long-term impact of the project from the point of view of all its stakeholders. The considerations presented in the article constitute an introduction to empirical research, primarily in the form of case-studies.*

Keywords: *Project management, Sustainable Development Goals (SDG), health care system, rare disease management, CLN2.*

JEL: *I10, P46, O22, Q49.*

Paper Type: *Research/case study.*

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1. Introduction

The paper presented here is a proposal to look at rare disease therapy as a project and apply the P5 GPM standard to define it. Numerous studies and reports (Chaos Report, Spałek, 2014; Madauss, 2000; Shenhar *et al.,* 2001; Hobbs *et al.,* 2011) indicate that one of the key factors for a project success is the proper, realistic definition of its purpose, scope and objectives. This therefore points to the special role of a project preparation stage (initiating, defining and then planning) in its final success.

At the same time, reports (international, e.g., WHO, EURORDIS, United Nation, The NGO Committee for Rare Diseases, EFPIA Patients W.A.I.T. 2019; 2020; 2021; and national, e.g., HTA Consulting Sp. Z.o.o., s.k.) on treatments for orphan/rare diseases point to numerous challenges in the implementation of such projects. These challenges relate to the negative impact of non-reimbursement of treatment on the patient and their family, the healthcare system as well as the economy.

As the consequences of these challenges have far-reaching social and economic effects, the authors proposed to define rare disease therapy projects using the P5 standard by Global Project Management. The main objective of the article was to present the benefits of using this standard to define social projects on the example of orphan disease therapy. This allowed the authors to consider three key perspectives for the correct definition of orphan therapy implementation projects - the social, economic and environmental perspective. The background to the discussion was the unequal access to therapy due to different reimbursement decisions in different European countries. The result of the experiment allowed to formulate scientific and practical conclusions and recommendations. At the same time, it allowed the authors to outline perspectives for further research in this area. In the article the term orphan therapy and rare disease therapy are synonymous/used interchangeably.

2. Project Initiation and Definition - The Essence of the Issue

Project definition is one of the key stages in the so-called project life cycle (Kerzner, 1984; Adamu, 2016; Oberholzer *et al.,* 2018). Its primary purpose is to formulate the rationale for the project, its purpose and scope, and to identify the key assumptions surrounding its implementation. Based on the information gathered during this phase, the organisation's management/project sponsor makes the decision to implement the project. The assumptions formulated during the definition phase will also accompany the project until its completion - mainly as a reference point, an initial idea of the objectives and benefits to be delivered by the project and the necessary resources and constraints. Consequently, this means that the quality of the project definition process will be strongly linked to the subsequent success of the whole project.

Project definition can take many forms and vary in scope, but it is possible to identify some universal issues that should be recognised in this phase. These are primarily:

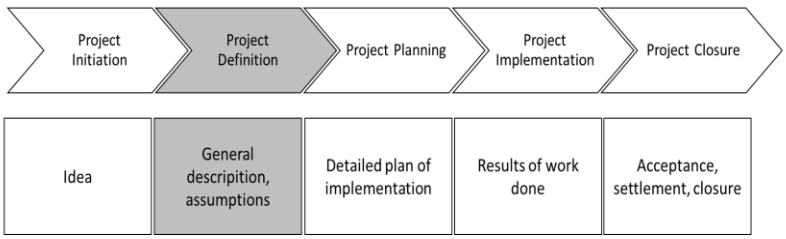
- reasons for undertaking the project
- project objective
- key results
- benefits resulting from its implementation (business case)
- estimated duration
- estimated resources, including financial ones
- preliminary risk analysis
- stakeholder analysis
- outline of the project team.

Other issues can also be indicated to be developed in the definition phase, which appear in some project management standards (i.a., PRiSM Methodology, Project Cycle Management / Logical Framework Approach):

- project impact on the organization's strategy
- dependencies with other projects
- project impact on the environment
- project impact on the society.

Figure 1 shows a general scheme of a project life cycle with a marked phase of project definition.

Figure 1. *Definition of the project in the project life cycle*



Source: *Own elaboration.*

Defining the project is of great importance to the key stakeholders of a project. In relation to key users, on one hand it allows for definition of requirements as to the results and benefits of the project, on the other hand - to make sure that they are properly interpreted by contractors. It also makes it possible to define the mechanisms for verifying the achievement of the project goal. Decision makers / sponsors, on the other hand, can reduce the risk of deciding whether to implement an unnecessary or unprofitable project through the definition phase. They can also make a comparative analysis between different ideas for achieving the same

benefits. Defining also allows them to pre-estimate the necessary resources and outlays that they will have to provide for the project execution time. In relation to a contractor, defining allows to determine the feasibility of the project, agree with the client / user the acceptance criteria.

In project management practice, there are many tools to support the definition phase. In a general way, they can be divided into general methods (also applicable in other areas of management, mainly supporting creative thinking) and comprehensive methods.

Table 1. *The most important methods to support project definition*

General methods	**Comprehensive methods**
● Introductory questions ● Checklists ● Assessment techniques ● Brainstorming / brainwriting techniques ● Expert techniques	● BOSCARD technique ● Project assumptions (Project brief / charter / statement / definition) ● Project canvas ● Feasibility study ● Business plan ● Business case ● Logical Framework Approach LFA

Source: *Own study based on Trocki and Juchniewicz, 2013.*

To sum up, the key challenges in the project definition phase are centered around answering the following questions:

- What benefits do we want to obtain by implementing the project?
- What are the project's results / outputs?
- What should be the scope of the project?
- What are the quality requirements for the project results / outputs?
- When should the project be completed?
- What project inputs and costs are accepted?
- Is the project purposeful, feasible and profitable (effective)?
- What are the project risks?
- Should you continue to work on the project?
- If so, what requirements should be applied for the project and how should they be documented?

3. The Specificity of Rare Diseases

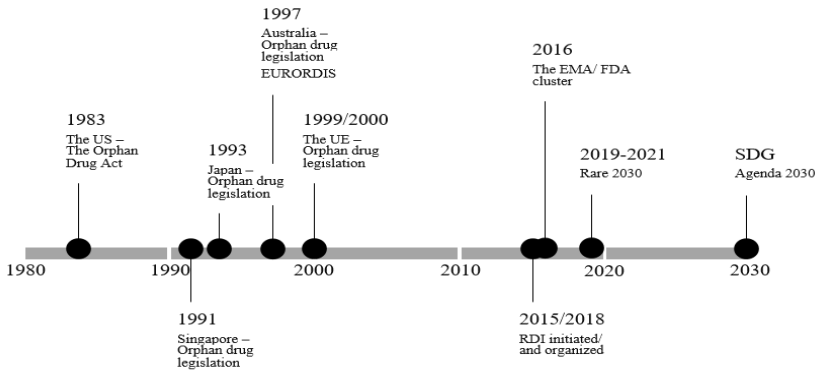
The definition of a rare disease focuses on its incidence and prognosis - the disease affects, according to the European standard, less than 5 people per 10,000. inhabitants, it is chronic, progressive and life-threatening. This criterion is met by at least 6 thousand diseases, while the number of diseases as yet undefined remains indeterminate. It is estimated that rare diseases affect 6-8% of the population, 300 million people in the world suffer from them, 30 million in Europe, and 3 million in

Poland (Kole, Hedley *et al.*, 2021). Due to the small scale of individual disease entities, knowledge about them, and thus the possibilities of helping patients, remain very limited. Diagnostic processes extend over time, in the vast majority of cases there is no causal treatment and patient care is intuitive and uncoordinated. This condition can be called an orphan phenomenon, just as a rare disease is called an orphan disease.

The chronic nature of rare diseases usually leads to disability, often multiple disabilities, and makes it necessary for third parties to care for the patient. Without causal treatment, patients die prematurely, and it should be emphasized that 70% of diseases begin in childhood (Kole, Hedley *et al.*, 2021). The lack of coordinated systemic support intensifies the effects of the disease. These difficult physical and mental experiences extend to the patient's immediate family, who "suffer from the disease" with him. The phenomenon of orphanhoo spreads to parents and siblings. As a consequence, it leads to the social and economic exclusion of many people, so it has multifaceted and multidimensional effects in the long run.

The awareness of the need for changes to equalize the chances of patients with rare diseases is growing (Figure 2). Especially in the last decade, the topic has involved leading global organizations such as WHO, UN and EURORDIS. The alliances are aimed at initiating large-scale projects to improve the situation of patients and their families in accordance with the fundamental right to health (Substantive issues arising in the implementation of the International Covenant on Economic, Social and Cultural Rights, General Comment No. 14, 2000); Palm *et al.*, 2013; EMA/ 633705/2016). This is a global challenge for sustainable development policy in the perspective of 2030 (Transforming our world: the 2030 Agenda for Sustainable Development, UN 2015), now also in a post-pandemic context (Resolution on the EU's public health strategy post-COVID-19, 2020).

Figure 2. *Milestones on the awareness axis for rare diseases*



Source: *Own elaboration based on "Report of the 2nd High Level Event Rare Disease Day at the United Nations", 2019.*

Management in the field of rare diseases - due to their specificity - involves many problems and requires the effort of many stakeholders (Ershadi *et al.*, 2019). The authors of the article focus on one of the key issues, which is the development of innovative therapies dedicated to orphan diseases. So far, they have been rare, because in market conditions they are unprofitable due to the high risk of failure, high production costs and a very limited sales market. Introduction by pharmaceutical market regulators of a system of incentives to work on projects addressed to this excluded group of patients is bringing results.

An important stimulus dynamizing research and production processes was granting the therapies - after positive three-stage verification - the status of an orphan drug [Regulation (EC) No 141/2000]. An orphan drug is the only or significantly more advantageous than the existing product intended for the diagnosis, prevention or treatment of a rare disease or other disease for which the marketing of the medicinal product will not generate a sufficient return on investment. In Europe, this status, in principle, provides the drug manufacturer with ten years of market exclusivity in the marketing of a medicinal product in a given indication [Commission Regulation (EC) No 847/2000; Regulation (EC) No 726/2004].

However, the successive development of the supply side does not solve the problem. There is still a shortage of drugs for 95% of diseases, and these orphan therapies, already invented, do not reach all those in need. The high price of drugs, which is also a consequence of rarity, discourages some countries from reimbursing them (Gałązka-Sobotka *at al.*, 2019; Pecyna, Wójcik, and Karus, 2019; Report on Rare Diseases availability of therapy: a review of solutions and recommendations for Poland, 2020). Eventually, this selective accessibility, depending on the place of residence, exacerbates inequalities in health and leads to the phenomenon of secondary orphanage. In the next part of the article, the authors discuss issues related to the specificity of defining health projects in the field of orphan drugs implementation from the perspective of national decision-makers.

4. Orphan Therapy Implementation Projects and the Challenges of Sustainable Development

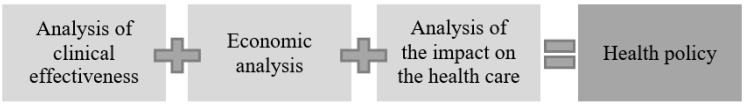
The presented situation in terms of solving the problems of insufficient availability of therapy for people suffering from rare diseases is largely conditioned by the high capital intensity of research on orphan drugs, and on the other hand, by a relatively small number of people using therapy, who are additionally territorially dispersed. This problem can be analyzed from different perspectives of individual stakeholders who undoubtedly have different, and sometimes even contradictory, goals. The authors proposed an analysis of the problem from the perspective of the payer, i.e., the national regulator that decides whether a given therapy will be implemented in the country. The authors analyzed both the process as well as the premises and methods used by the decision-maker to assess the possibility of implementing the

therapy, on the other hand, they proposed additional premises that could be taken into account when making the decision to implement.

The implementation of this type of health projects undoubtedly requires a special approach to project initiation and definition, because the environment of health projects is extremely complex. It covers both legal, organizational and economic factors, but also issues of social justice and exclusion (Report from Rare Disease Day®, 2019).

Orphan drug technologies do not fall within the standard framework of assessment for reimbursement decisions (Health Technology Assessment, HTA; Health Technology Assessment Guidelines, AOTMIT, Warsaw 2016), mainly due to poor clinical statistics (Evidence-Based Medicine, EBM). The price is usually high and exceeds the Qaly limit (three times GDP per capita in Poland). It therefore significantly reduces the payer's limited budget, who could redirect funds to a larger group of patients suffering from more common diseases. Orphan drugs raise doubts at the level of all analyses: clinical effectiveness, economic efficiency and impact on the health care system (Figure 3). On the other hand, orphan drugs are a response to the current discrimination, urgent need and pressure to deliver drugs in a rapidly progressing disease, for which there is usually no alternative to help.

Figure 3. *Elements influencing the country's health policy process*



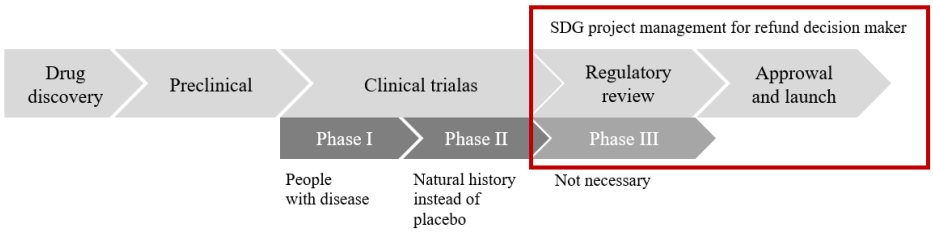
Source: *https://www.aotm.gov.pl/useful-oceny-technologii-medycznych.*

The compromise in this situation is the redesign of the clinical trial model and the parallel conduct of the last phases of research on formally treated patients (Figure 4). Drug assessment and approval agencies recognize the specificity of rare diseases and accept the small sample size and the lack of randomized and placebo-controlled trials, EMA has implemented a fast track registration of PRIME: priority medicines (Development support and regulatory tools for early access to medicines, 2016, EMA / 531801/2015). The reimbursement regulations make it possible to deviate from the Qaly rule, also in Polish legislation. Here the progress is noticeable, but there are no target system solutions. The national plan for rare diseases, which has been in operation in other European countries for several years, is still under development. The lack of a strategic framework and dedicated procedures is an impediment, but not a blockage. Budget constraints are the main problem.

Operational solutions in the form of public fundraising for treatment of individual patients (out of pocket) only emphasize the weakness of the system, besides, they are possible in the case of one-off, often spectacular aid actions, and not in the case of

chronic treatment (Baumbusch *et al.*, 2018; Gombocz and Vogler, 2020). A chance for changes in this area is the Medical Fund established in 2020 with a target annual budget of PLN 4.2 billion, which will finance early and conditional access to drug technologies with high clinical value and access to drug technologies with a high level of innovation (Act of October 7, 2020 on the Medical Fund, Journal of Laws of October 26, 2020, item 1875).

Figure 4. *Drug implementation process - specificity of orphan technology and scope of the project for refund decision maker*



Source: *Own study based on https://www.ifopa.org/clinical_trial_phases.*

The need for a different approach to the assessment of orphan drug technologies focuses the efforts of experts on creating tools that take into account this diversity and realistically support the reimbursement decision-making process, taking into account the conflicting interests of all stakeholder groups. Many countries implement their own solutions, but they all take a broader view and create a spectrum of criteria that are important primarily from the patient's point of view, e.g., the availability of alternative treatment options, the impact of the disease on life expectancy or the impact on the functioning of the family (Multi-criteria decision analysis (MCDA), MCDA Consortium. Multi-criteria Decision Analysis in the evaluation of technologies used in non-oncological rare diseases. Warsaw 2020. Poland).

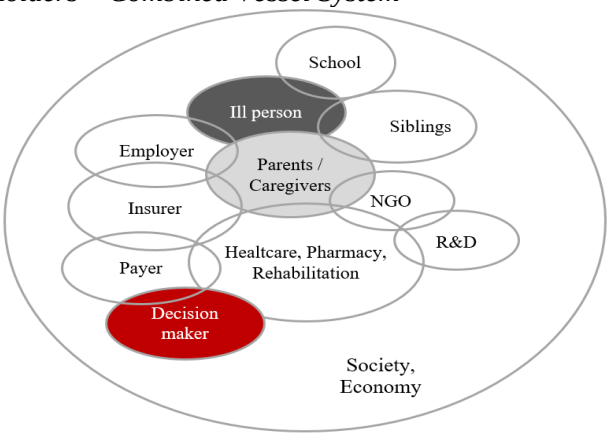
MCDA broadens the horizon of perceiving the problem, it also signals issues related to the impact of the disease on the environment, which increases the chances of balancing the influence of many stakeholders on the reimbursement decision. This is a direction that, according to the authors of the article, should be developed by supplementing the analyzes with far-reaching long-term effects of the unavailability of treatment. Lack of access to a drug in a given country compared to the existing aid in other countries is a special case here. Looking at orphan drug technology through the prism of sustainable project management creates such opportunities.

Rare disease treatment involves a large group of interrelated stakeholders (Figure 5). They all have an impact on the implementation of sustainable development goals in the field of rare diseases and are at the same time beneficiaries of the actions taken in the short and long term. Public funds enabling reimbursement are limited, and a

positive reimbursement decision should not be included in the analyzes only on the expenditure side. Treatment undertaken by the patient generates a number of benefits, which will also be reflected in budget revenues.

Therefore, a negative decision saves money only in the short term. An additional significant dimension of socio-economic losses appears along with the phenomenon of the so-called "Health emigration" - the patient goes abroad to live and start treatment in a country where the therapy is reimbursed due to the lack of such possibilities in the home country. For example, in Poland, 2-6 new cases of the ultra-rare CLN2 (childhood dementia) are diagnosed annually, of which 2-5 children emigrate with their families, mainly to Germany. These are only estimates, as there is no consistent national patient registry.

Figure 5. *Stakeholders – Combined Vessel System*



Source: *Own study.*

The lack of reimbursement of treatment has direct consequences for the patient, his caregivers (usually parents) and healthy siblings:

- deteriorating health excludes the patient from education, professional development (if the patient reaches adulthood at all) and social life;
- exclusion also affects the caregivers of the patient, one of the parents (most often the woman) quits his/her job, and for the other caregiver professional development is difficult, because the ill member of the family means a number of limitations, more responsibilities and less time;
- there is also a syndrome of a healthy sibling of a sick child who lives in the shadow of his sister or brother's illness and experiences many limitations;
- physical effort, mental stress, constant fear, a sense of helplessness and lack of understanding, life “next to” a normal world, in Poland, without and effective system support is destructive for the whole family;
- "health emigration" is a difficult decision, usually taken under time pressure, "snatching" the family from the current environment, sometimes its

temporary separation, the need to change jobs, in most cases lowering the standard of professional and social life in the initial phase of functioning in emigration, a huge mental burden.

A negative reimbursement decision also adversely affects the national health system:

- costs of symptomatic treatment, which are long-lasting and increase as the disease progresses;
- lack of development of the medical potential and competence in the field of a given drug technology and a wide range of products and services supplementing a treatment (rehabilitation, equipment supporting independence, etc.);
- limited awareness of a given disease, and thus a slower diagnosis of subsequent cases and a reduction in the effectiveness of treatment (in the case of implementing a therapy available abroad);
- limited possibilities of gathering a knowledge base, experience, good practices, which results in stagnation in the development of R&D sphere in this area;

The lack of active measures in the implementation of rare disease therapies also affects the long-term economic development of the state:

- exclusion of ill person and family members from the labor market, lower productivity, tax losses, the need for social assistance for these families;
- the intensification of "health emigration" phenomenon means a drainage of human resources and a negative impact on demographic indicators of a given country - the whole family, including healthy siblings, eventually emigrates with the patient; in a new place of residence all of them, after receiving systemic support, begin to function again, both professionally and socially;
- emigration extinguishes the need to support patients, thus wasting the possibility of changes and development in the area of a given disease entity, therefore in the home country the trend of increasing effectiveness of the health care system through patient empowerment is disrupted, as the patients' potential, knowledge, experience and commitment to improve the situation of the ill persons disappears (Władysiuk, Libura, Rolska-Wójcik, and Plisko, 2020; 2021);
- problems with orphan drugs reimbursement cause that a given country is perceived by the participants of the process as a partner less efficient, committed and open to innovation.

The area of rare diseases, due to a small number and spatial dispersion of patients, requires special supranational, even global cooperation. Incentive mechanisms in research and production remain ineffective in the absence of reimbursement of treatment with orphan products. A broad international horizon is also necessary in

terms of developing optimal solutions as to the pricing policy of these drugs, in line with the WHO's call for fair, transparently justified prices (Medicines reimbursement policies in Europe, WHO 2018; Degtiar, 2017). Otherwise, the limitation of the possibilities of helping the sick and the disproportions between countries will deepen.

A wide impact of lack of availability of treatment for rare diseases and their social impact, as well as the perception of the country's ability to care for patients, but also lost benefits in the area of development of a broadly understood sphere of health protection, indicates that it is necessary to extend the analysis of the environment and project profitability to include project analysis in the context of sustainable development.

5. P5 Standard

The reference of sustainable development to project activities was developed by Green Project Management Global (GPM Global, 2021) as the P5 ™ Standard for Sustainable Project Management (PRiSM). The study addresses three measurable elements of sustainable development, the social aspect (People), the environmental aspect (Planet) and the economic aspect (Profit) along with the process aspect (Management) and the product (Technical) aspect of the project. The name of the P5 ™ standard means: Product, Process, People, Planet, and Prosperity (The GPM P5 ™ Standard, 2019).

The P5 standard is a summary of actions that are taken by a project manager and a project team to reduce the negative impact on sustainable development and increase the positive impact. P5 Standard provides a checklist of topics to consider in terms of the impact of individual project activities on project results (Carboni *et al.*, 2016, 2020; Trocki 2019).

The main purpose of P5 standard is to identify potential impacts on sustainability, both positive and negative, that can be analyzed and communicated to management to support a conscious decision making and efficient allocation of resources. The ontology of P5 standard is shown in Table 2 below. An ontology helps to deal with complexity by organizing available information in a coherent way (The GPM P5 ™ Standard, 2019).

Table 2. *P5 ontology*

<table>
<tr><td colspan="6">PROJECT</td></tr>
<tr><td colspan="3">Product Impacts</td><td colspan="3">Processes (Project Management) Impacts</td></tr>
<tr><td>Lifespan of Product</td><td colspan="2">Servicing of Product</td><td>Effectiveness of Project Processes</td><td>Efficiency of Project Processes</td><td>Fairness of Project Processes</td></tr>
<tr><td colspan="2">People (Social) Impacts</td><td colspan="2">Planet (Environmental) Impacts</td><td colspan="2">Prosperity (Economic) Impacts</td></tr>
</table>

- Labor Practices and Decent Work - Society and Customers - Human Rights - Ethical Behavior	- Transport - Energy - Land, Air and Water - Consumption	- Business Case Analysis - Business Agility - Economic Stimulation

Source: *GPM Standard P5 ™ for Sustainable Project Management, GPM Global Version 2.0, GPM EMEA Sp. z o.o, Kraków 2021, p. 3.*

The P5 standard is a tool that allows to analyze the impact of a product impact, as well as the impact of project management processes on individual areas related to the impact on society, environment and economy. The possible impacts assigned to the areas are related to the Sustainable Development Goals. Therefore, this tool gives a real opportunity for a structured implementation of sustainable development in the processes of both product production and exploitation and management processes in the project.

The implementation of the principles of sustainable development in projects is a big challenge for project managers. These difficulties are related both to the incomplete knowledge of project teams and other stakeholders and the very integration of the principles of sustainable development with the practice of project management (Ozumba *et al.*, 2020; Maltzman *et al.*, 2016). Therefore, the possibility of applying the P5 standard can be a significant facilitation for project managers and management boards of the organization. Using P5, managers can identify in the first phase the project's possible impacts, link them to the SDGs and then, by analyzing them, identify those they intend to focus on during the project implementation and product maintenance.

The selection process is related both to risk assessment of a negative impact of processes and the product on society, environment and economy, as well as to the organization's strategy. This awareness makes it possible to distinguish those SDGs that are most important for the organization and for the project stakeholders. Developing such a linkage matrix supports the SDG prioritization process for the organization.

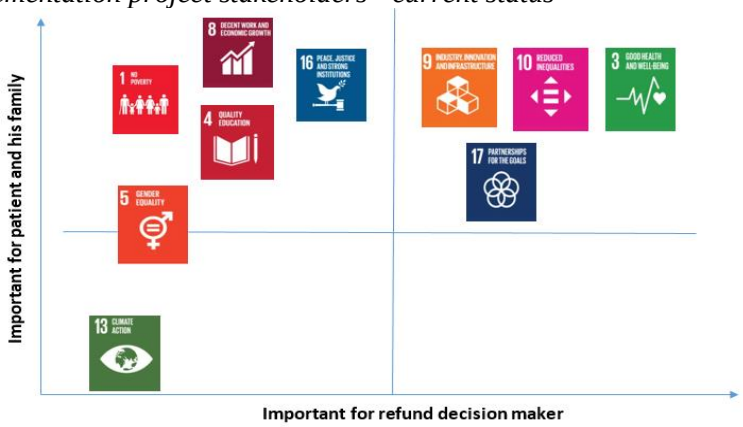
6. Proposal / Applicability of P5 to Define Rare Disease Therapy Projects

The authors have presented a proposal to define a project for the implementation of a rare disease treatment in a sample country, based on the example of Polish legal regulations using the P5 standard. The legal conditions for drug implementation are defined in Poland and their general outline is presented. An issue that is still under discussion and is a challenge is how to analyze the profitability of such an implementation. In the authors' opinion, the traditional methods and techniques used in the preparation of the Business Case are insufficient.

A drug deployment project for rare diseases requires additional stakeholder impact analysis linked to the SDGs. The analysis started with determining which

Sustainable Development Goals should be included in Business Case for orphan drug implementation projects. It seems natural that we see greatest impact in People and Prosperity areas. Figure 6 below presents reference to sustainable development goals in relation to key stakeholders is presented in this Figure.

Figure 6. *Sustainable Development Goals in relation to the interest of the orphan drug implementation project stakeholders - current status*



Source: *Own study.*

The following goals can be identified in the area of greatest interest of key stakeholders, i.e., both the drug payer and patients and their families:

- Goal 9: Build resilient infrastructure, promote sustainable industrialization and foster innovation,
- Goal 10: Reduce inequality within and among countries,
- Goal 3: Ensure healthy lives and promote well-being for all at all ages,
- Goal 17: Revitalize the global partnership for sustainable development.

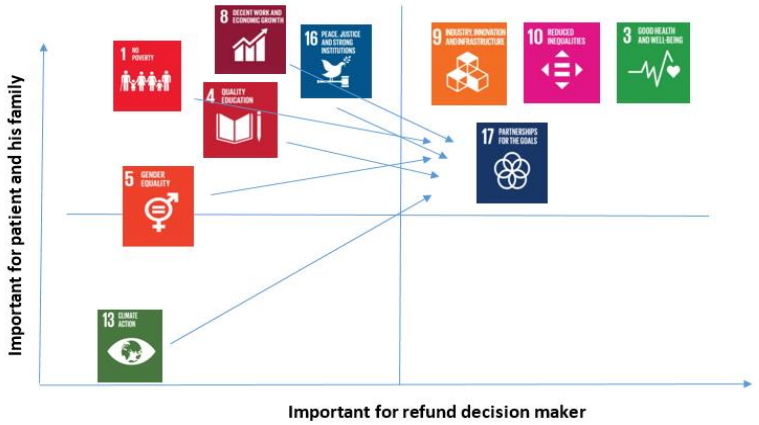
It seems that the indicated goals are linked by interests of both the payer of the drug and the patient and their immediate environment. Therefore, these goals can be addressed in the analysis of effectiveness assessment of orphan drugs implementation in Poland. The conducted analysis has also shown that there are goals that are important for the patient and his family, but in our opinion these issues are not sufficiently taken into account by the payer. These are:

- Goal 1: no poverty,
- Goal 4: quality education,
- Goal 5: gender equality,
- Goal 8: decent work and economic growth,
- Goal 16: peace, justice and strong institution.

In addition, Goal 13 Climate Action is worth mentioning, which should be important for the state, and can be implemented by reducing the necessary logistics related to the movement of the patient for treatment to remote places, e.g. other countries,

continents or to the only center treating a given disease in the country. These are objectives, the inclusion of which would certainly improve the quality of the payer's "offer" in the field of rare diseases therapy, and, consequently, the positive impact of this type of projects on 5 elements of sustainable development.

Figure 7. *Sustainable Development Goals in relation to the interest of orphan drug implementation project stakeholders - desired state*



Source: *Own study.*

In the authors' opinion, P5 standard described in the previous section could provide significant support in the process of defining by the payer rare disease therapy projects. As already mentioned, the individual components of the model are related to the selected SDGs, which allows, in the context of the considerations in this article, to analyze which of these elements could most help to include SDGs 1, 4, 5, 8, 13 and 16, as shown in Figure 7.

Additionally, the authors presented the possibility of using P5 analysis to define orphan drug implementation projects. Table 3 shows the individual goals of sustainable development that should be taken into account in estimating the benefits of implementing an orphan drug. Some of the indicated goals are identified by both the patients and the decision-maker as important (Figure 6), while some of them are important only for particular groups or are ignored by both the decision-maker and the patient, i.e., Goal 13. In Table 3, the last two groups have been bolded.

Table 3. *Proposal to use P5 for an orphan drug implementation project*

PROJECT				
Product Impacts		Procesess (Project Management) Impacts		
Lifespan of Product	Servicing of Product	Effectiveness of Project Processes	Efficiency of Project Procesess	Fairness of Project Procesess
People (Social) Impacts		Planet (Environmental) Impacts	Prosperity (Economic) Impacts	

- Labor Practices and Decent Work: ● **Goal 8 Decent Work and Economic Growth,** ● Goal 3 Ensure healthy lives and promote well-being for all age, ● Goal 10 Reduce inequality within and among countries, ● **Goal 4 Quality education** - Society and Customers: ● Goal 10 Reduce inequality within and among countries ● Goal 17 Revitalize the global partnership for sustainable development - Human Rights: ● **Goal 5 Gender Quality** ● **Goal 16 Peace, justice and strong institutions** ● **Goal 8 Decent Work and Economic Growth** - Ethical Behavior: ● **Goal 16 Peace, justice and strong institutions**	- Transport: ● **Goal 13 Climate Action** ● Goal 9 Built resilient infrastructure , promote sustainable industrialization development and foster innovation	- Business Case Analysis: ● **Goal 1 No Poverty** ● Goal 9 Built resilient infrastructure, promote sustainable industrialization development and foster innovation - Economic Stimulation**:** ● **Goal 1 No Poverty**

Source: *Own study.*

7. Summary

In the authors' opinion, rare disease therapies can and should be treated as projects, and therefore it is advisable to use tools developed for project management in their implementation. At the same time, these are extremely complex, multi-faceted projects with costs and benefits that are difficult to measure. This means that they require special attention in the definition phase, during which the key assumptions, analyses and measurements are created. Practice shows that HTA analysis is used to define rare disease therapies, and that analysis focuses on proving clinical effectiveness, costs of therapy and its impact on the payer's budget, like for any other therapy. Expert and patient communities emphasize the need to broaden the horizon of analyses for orphan therapies, e.g. by using multi-criteria analysis that will help to include a broader spectrum of costs and benefits.

In the authors' opinion, the analyses should capture such issues as: costs of professional and social exclusion of the patient and his family, and if therapy is available in other countries, the costs of family emigration, change of place of residence and / or work. The issue of spatial accessibility is also important, too sparse a network of treatment centers generates unnecessary travel costs, wasted time or regular exclusion from work. All these issues are directly related to sustainable development under the so-called SDG - developed by the United Nations. These are important issues for both patients and their families and decision makers (as shown in the article).

The aim of the article was to propose the P5 GPM standard as a tool that allows all participants of rare disease therapy to increase their awareness of sustainable

development issues and take them into account to a greater extent in the process of defining such projects.

Due to the nature of the article, it has some limitations. These include, first of all, the narrowed scope of the analysis, and the lack of expert panels that would verify the authors' proposals. This means that the concept requires a broader discussion and further research on the possibility of using P5 in other similar projects. In the authors' opinion, it would be valuable to conduct a study among the health care system stakeholders, including patients and patient organizations, decision makers (government), payer, service providers, manufacturers of medical technologies, which would allow to determine the chances of promoting the tool, making participants of rare diseases therapy aware of the broad definition of these initiatives.

However, it should be clearly emphasized that apart from building awareness, competences and promoting tools, there is a need to change the regulations in this area. In Poland, the procedure for adopting the Plan for Rare Diseases is currently underway (Resolution of the Council of Ministers on the adoption of the document Plan for Rare Diseases. Project dated 27/04/2021), which will be a response to the recommendations of the European Union from 2009 (Decision No. 1350/2007/EC of the European Parliament and of the Council; Council Recommendation 2009/C 151/2). At the same time, the draft amendment to the Reimbursement Act is being consulted (Act amending the Act on reimbursement of drugs..., Draft of June 30, 2021).

While the Plan meets many demands of communities related to rare diseases, the draft amendment to the act seems to omit many important issues, especially the participation of patients in key decision-making processes. It also poses a threat to the reimbursement of orphan therapies that will not be included in the list of innovative drugs financed by the Medical Fund. This is because the amendment provides for an obligatory refusal to cover a drug if the cost of obtaining an additional year of quality-adjusted life exceeds six times the GDP per capita. The discussion on the topic is therefore necessary and should connect all sides of the problem.

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