White Paper

Innovative Funding Models for Treatment of Rare Diseases

A global study of new financing solutions for rare diseases

COMMISSIONED BY ROCHE
PREPARED BY IQVIA

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Executive summary

Rare diseases (RD) have inherently small and dispersed patient pools, which often put them at a low priority for governments. Historically, this has led to insufficient shares of national health budgets worldwide being allocated to treat rare diseases. Due to this, the associated funding challenges for rare diseases bring continuous and increasing financial impact for patients, health systems and economies and, without action, will only grow.

As funding for RD is a major challenge globally, with significant unmet needs, collaboration across health systems is vital to resolve the difficulties of deterring sustainable treatment access. IQVIA and Roche, in partnership, have identified the challenges faced in RD access and key case studies to help develop strategic funding and financing solutions for the treatment of rare diseases.

Globally, there are several initiatives specifically for addressing funding challenges for access to treatment of rare diseases. These involve partnerships across the RD community to better understand countries' RD funding landscape, support the development of funding solutions, deliver innovation at a lower cost to society, and ultimately drive access for RD patients.

This paper was commissioned by Roche, and carried out a series of research studies focused on understanding the characteristics of the funding models available worldwide for therapies in rare diseases. For this purpose, IQVIA and Roche looked at a subset of 40 countries distributed across regions: Africa, America's, Asia Pacific (APAC), Europe, Middle East and Latin America (LATAM). The research primary focus was on identifying those financial models that could increase patient treatment access.

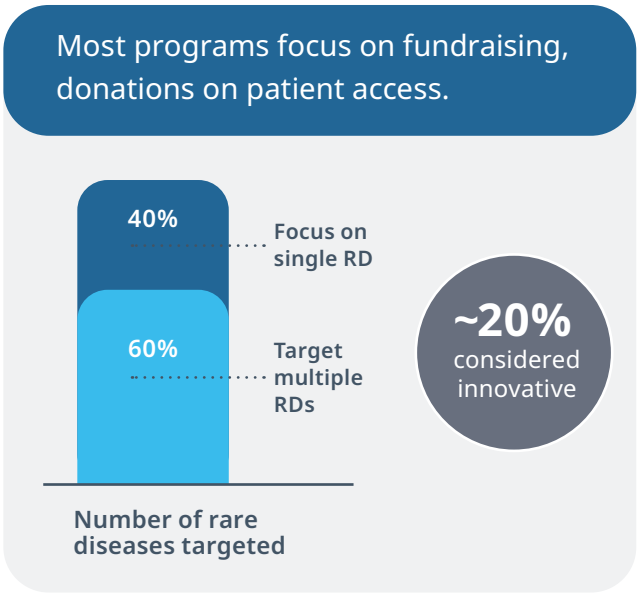
Results

Several challenges were identified in many countries around the allocation of funds and many of them do not have robust rare disease strategies and legislation to ensure continuous provision of funds for rare diseases. Through a combination of literature review, in-country experts and healthcare expert interviews, 134 diverse global rare diseases funding case studies across more than 40 countries were identified. The majority of funding initiatives were found to be concentrated in Europe and North America, with half of the case studies identified coming from countries in these regions. APAC and Latin America cases account for 18% and 16%, respectively. With regard to the breadth and scope of the models, many of them are considered "small" based on the number of patients and fund size.

134 global rare disease case studies across 40 countries

~50% from countries in North America & Europe

Many considered "small" based on number of patients fund size



Source: Combination literature review, expert interviews

Results show that most funding programs address more than one rare disease, with ~60% targeting multiple RDs and 40% focusing on single RDs. Across all markets, most programs focus on fundraising, donations or patient access programs, and only ~20% of funding programs can be considered innovative (e.g. private insurance, financial instruments or government interventions). This indicates that in comparison with other non-communicable diseases (NCDs) such as oncology, in the space of rare diseases, there is a need to launch more sophisticated funding models to unlock new sources of funds that would ensure the sustainability of solutions.

Nonetheless, there are some examples of funding models showing innovation in resource mobilisation and/or pooling. Examples include public-private resource pooling alliances, sales-linked donations and fundraising partnerships. For instance, in Scotland, the New Medicine Fund provides access to orphan drugs similar to the Cancer Drug Fund in England. Recently, Singapore established a new Rare Disease Fund specifically to support rare disease patients. This fund leverages a combination of donations and government-matched contribution to help Singapore Citizens with specific rare diseases who require treatment with high-cost medicines.

The challenge for policymakers and rare disease stakeholders such as patient organisations and the private sector will be to identify funding solutions most appropriate for their health system and the population's needs and ability to sustainably adopt them. It is also a priority to build adequate evidence for rare disease funding prioritisation and accompany it with strategic guidance on approaching funding and financing solutions. In addition, it will be crucial to focus on identifying best practices and key learnings of similar international experiences and identifying external experts that can support discussions with key decision makers to create the required innovative solutions.

Partnerships will be critical to creating holistic programs focused on more than one rare disease area. Patient groups, providers, NGOs, foundations, biotech companies, and physician communities are key influencers for policymaking and can be potential partners for funding and implementation.

The world post-COVID-19

On top of the known healthcare challenges, the COVID-19 pandemic is putting health systems under tremendous pressure to meet population health needs. While the full impact of the COVID-19 pandemic is yet to be determined, significant resources are currently being diverted to meet COVID-19's intensive demands on hospitals, healthcare providers and vaccination campaigns.

Although it is hard to accurately predict the effects of COVID-19, in the mid-term, health systems budgets are expected to tighten, making it harder to deploy additional funds to previously unprioritised areas. The focus will most likely be on health for the masses and not on targeted or individual therapeutic options. Some of these

situations are not new for rare diseases but to change it will demand a new approach and new partnership models that will help reimagine healthcare delivery, financing, and access to innovation.

In the backdrop of COVID-19, securing access to rare diseases and maintaining these objectives will demand for non-traditional collaborative approaches, increasing efficiency gains and funding solutions from public and private institutions to appear in a greater capacity.

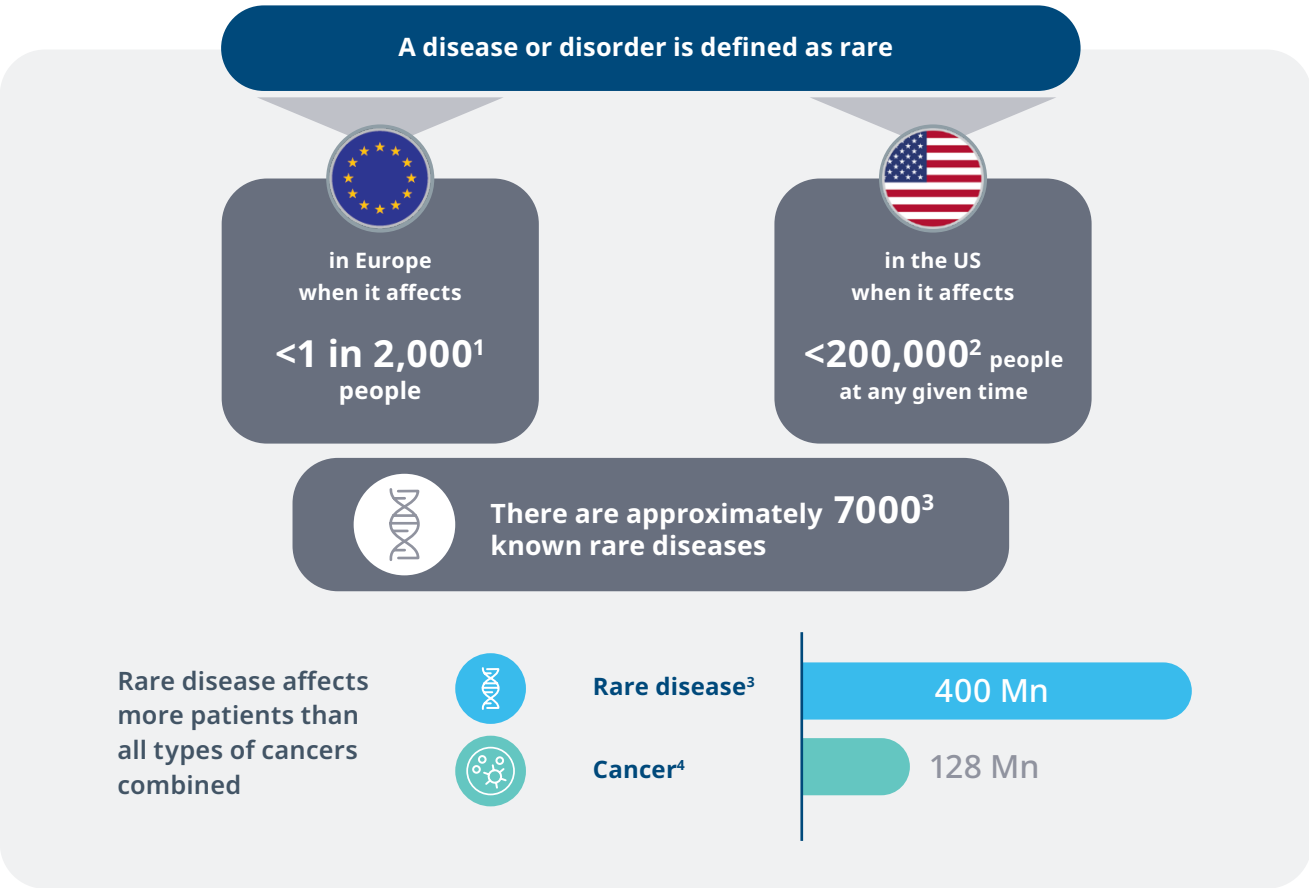
Introduction

Rare disease overview

A rare disease is a disease that affects a small percentage of the population. There is no global consensus on the definition, and these vary across different regions and countries. For this report, we limited our scope to rare diseases without support from additional funding sources, thus excluding rare cancers, whose patients benefit from cancer funding.

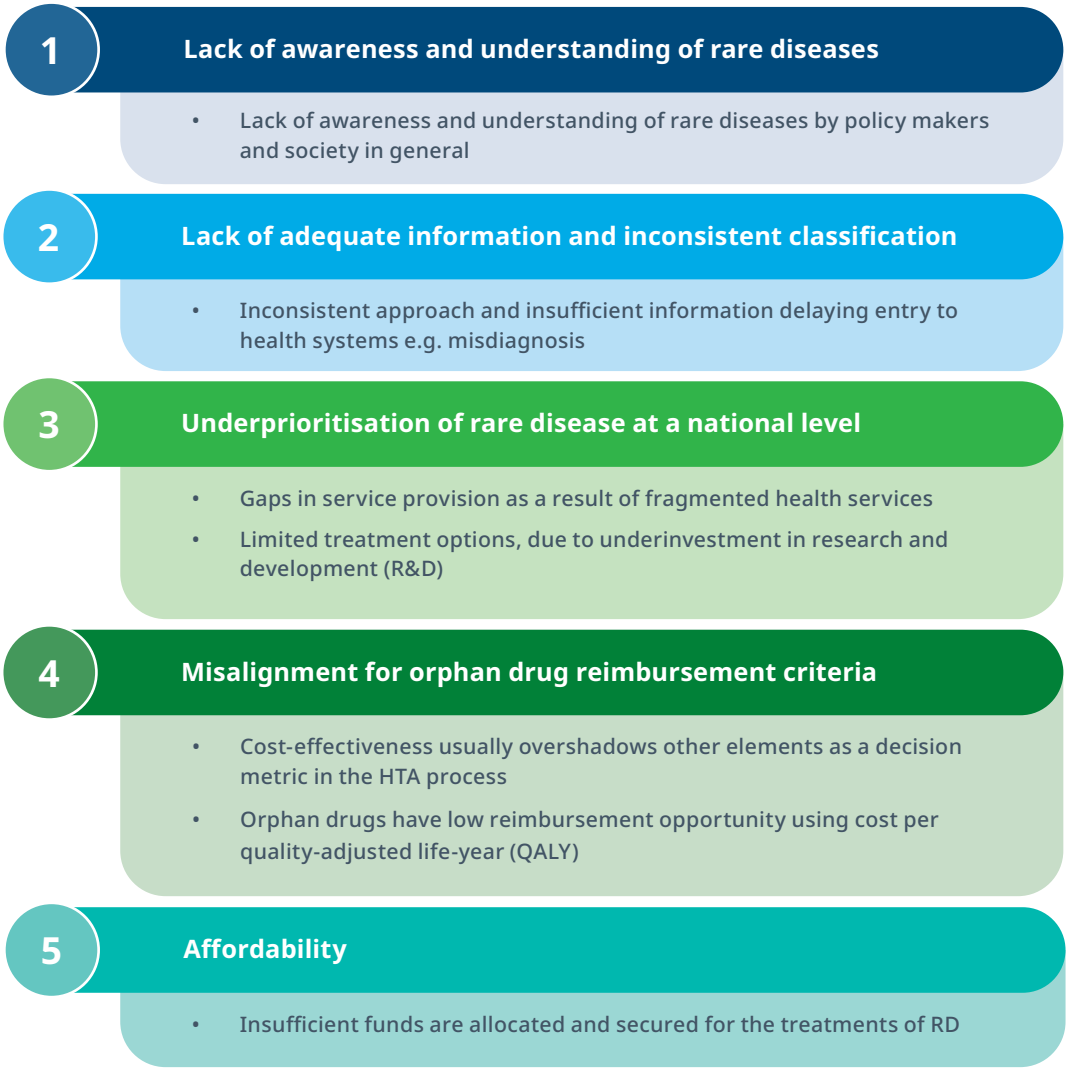
While each rare disease may only affect a small number of people, collectively, they affect more people than all types of cancer combined, as shown visually in Figure 1.

Figure 1. Global rare disease definition and prevalence



An inherent challenge for a disease affecting only a tiny percentage of the population is there are more complex funding and access barriers than common diseases. The small and dispersed patient pools have caused rare diseases to be a low priority for the government and often even pharmaceutical companies, leading to insufficient shares of national health budgets and research allocations.

Many rare diseases lack curative therapies, highlighting the need for improved coordination to ensure that patients have access to available treatments and services. Key stakeholders such as governments, public health agencies and medical research communities are faced with challenges at a policy level:



The combination of these challenges often leaves patients exposed to a choice between financial catastrophe or abandoning treatment.

Collaboration across the healthcare system is required to deal with these systemic challenges. For example, the provision of training and awareness building among frontline healthcare workers to recognise rare diseases and direct patients to effective treatments. Failing to address rare diseases at a policy and systemic level will lead to the neglect of society's most vulnerable people, leaving them to suffer chronic debilitation, often right from childhood, and leaving a huge impact on families and carers.

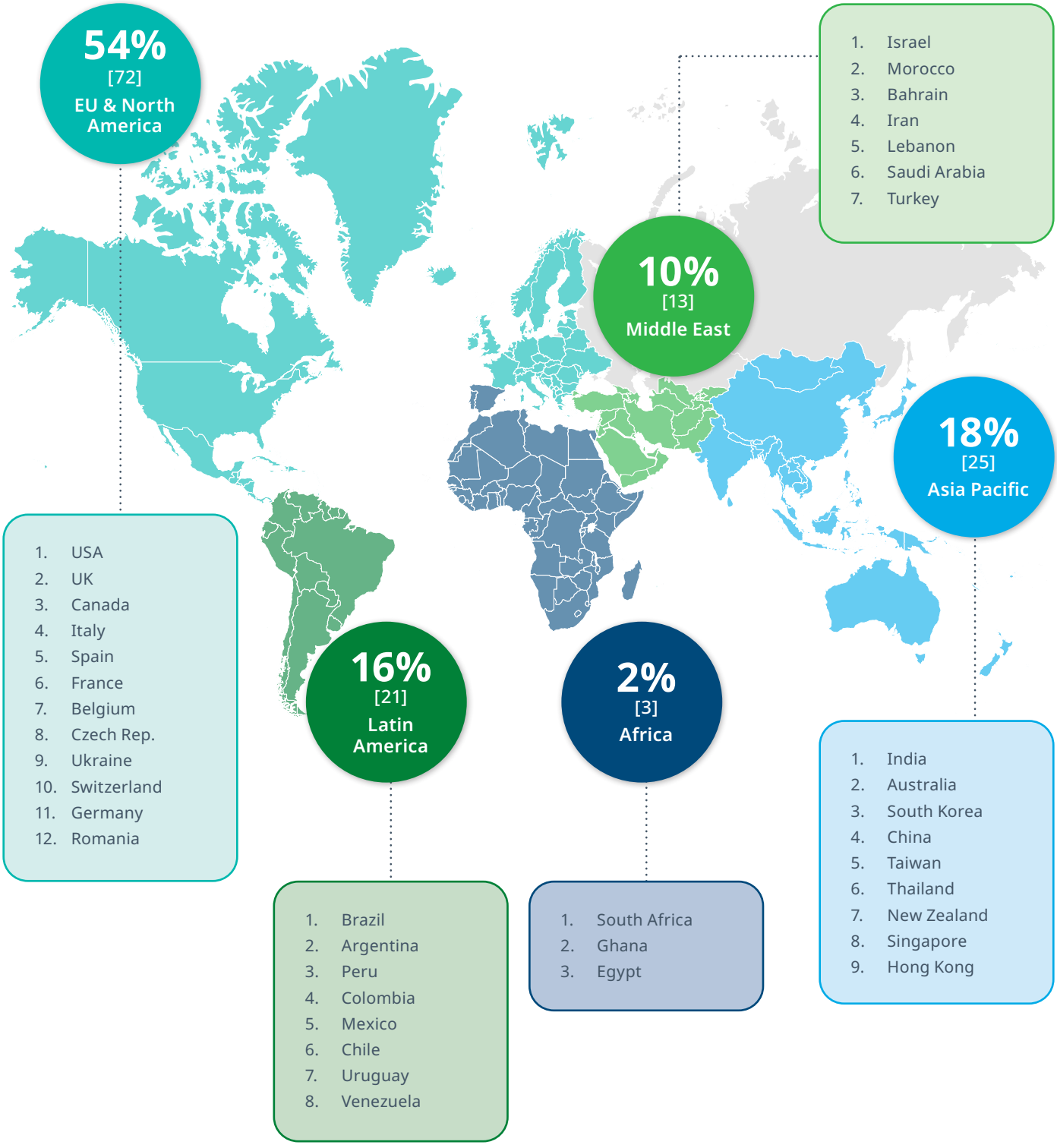
In the past two decades, many countries have taken action to tackle the problems mentioned above, but there is still room for improvement. National innovative funding must be sustained and expanded to address these complex funding gaps effectively.

Study results

Countries involved in the study

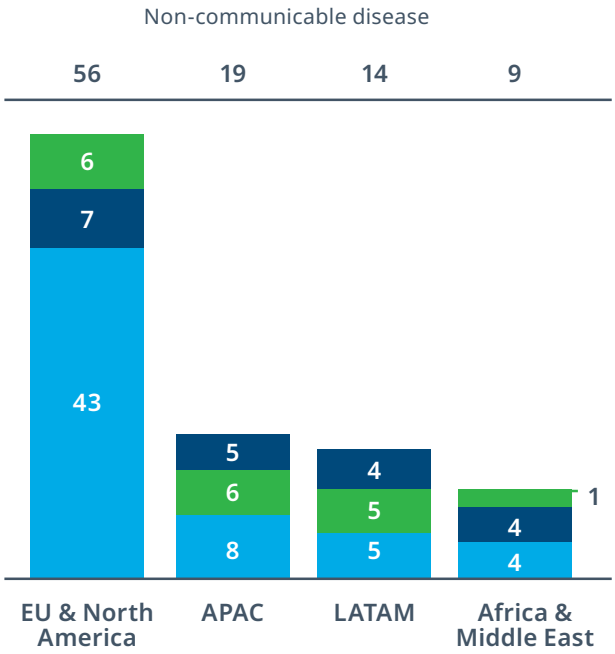
This study focuses on understanding the characteristics of innovative funding models that have been leveraged worldwide to increase access for rare diseases. As part of the research, case studies were identified across more than 40 countries, as shown in Figure 2.

Figure 2. Countries with funding models identified



The majority of the funding mechanisms identified in this study were found in North America and Europe, accounting for just over half of the models. The remaining cases can be traced to three blocks, with APAC and LATAM having a similar share of initiatives and the Middle East together with Africa showing a smaller representation.

Figure 3. Breakdown of rare disease funding program types per region and degree of sophistication



Degree of sophistication		Program archetypes
Low	• Operate on traditional donation-funding models	• Donation • Fundraising
Medium	• Moderate diversification of funds, time and capacity to implement	• Patient access programs (PAPs)
High	• Diversified funds, requiring substantial time and capacity to implement	• Reimbursement agreements • Goverment intervention • Financial instruments • Private insurance

The distribution for the prevalence of funding mechanisms is consistent with findings from the **Funding Environment for Rare Diseases in Low and Middle Income Countries (LMIC) report** on the status of RD coverage in ten LMICs, where it showed that many developing economies shared multiple challenges associated with RD management including underinvestment and underprioritisation of these conditions. Nonetheless, as shown in Figure 3, the share of highly innovative models as a percentage of total funding mechanisms is the highest in APAC and LATAM.

"Collaboration across the healthcare system is required to deal with these systemic challenges.... Failing to address rare diseases at a policy and systemic level will lead to the neglect of society's most vulnerable people."

Rare disease funding solution library

To better understand the different types of rare disease funding programs, the innovative funding models framework introduced in the **Innovative Funding Models for High-Cost NCDs report** helps to characterise innovative funding and financing based on the component of the model. Figure 5 features the five main archetypes (found on the following page).

As part of the research for this study, a rare disease library with the identified 134 various global rare disease funding case studies was created. As mentioned, most case studies originate from the Europe and North America and can be considered small in scale (by the number of patients and value of funds). Figure 4 shows a visual breakdown of the type of case studies categorised by the program archetypes.

Figure 4. Breakdown of rare disease funding program types

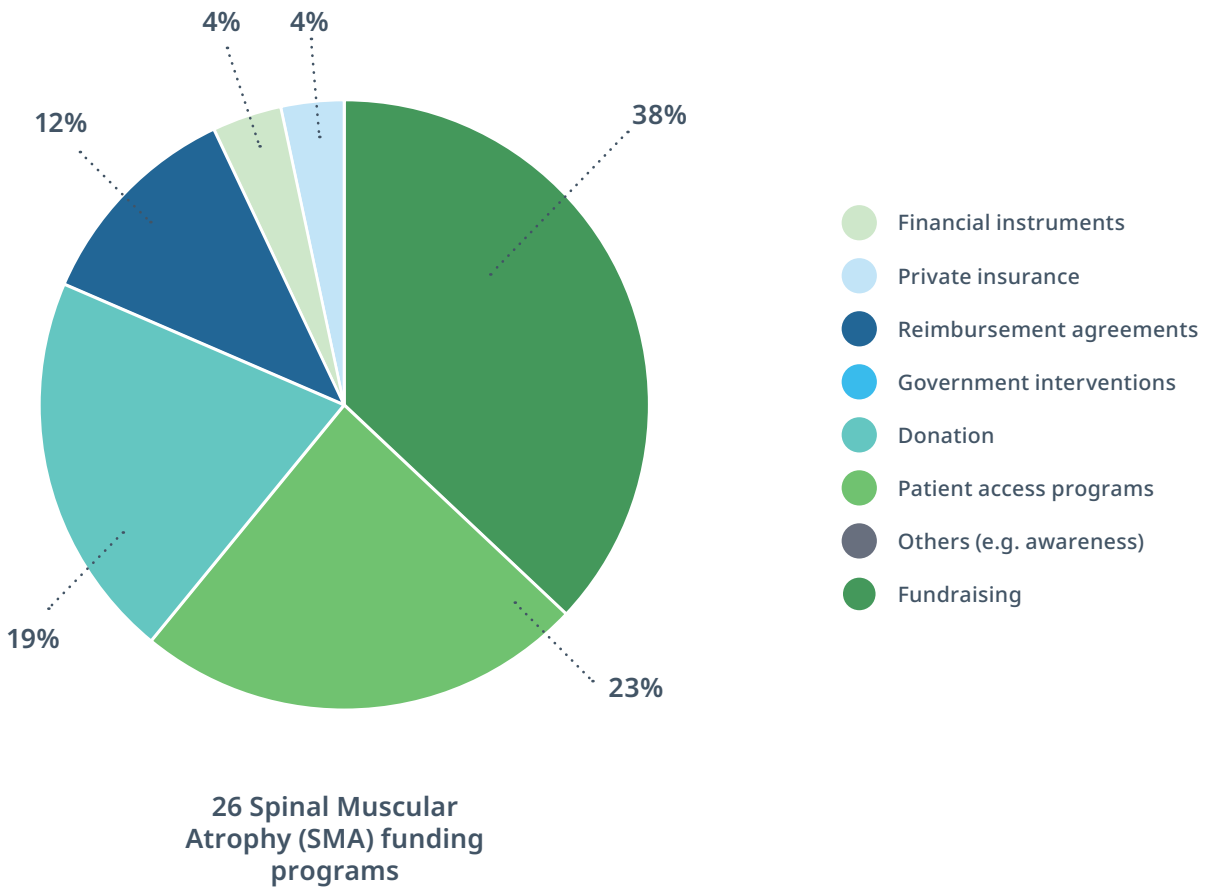
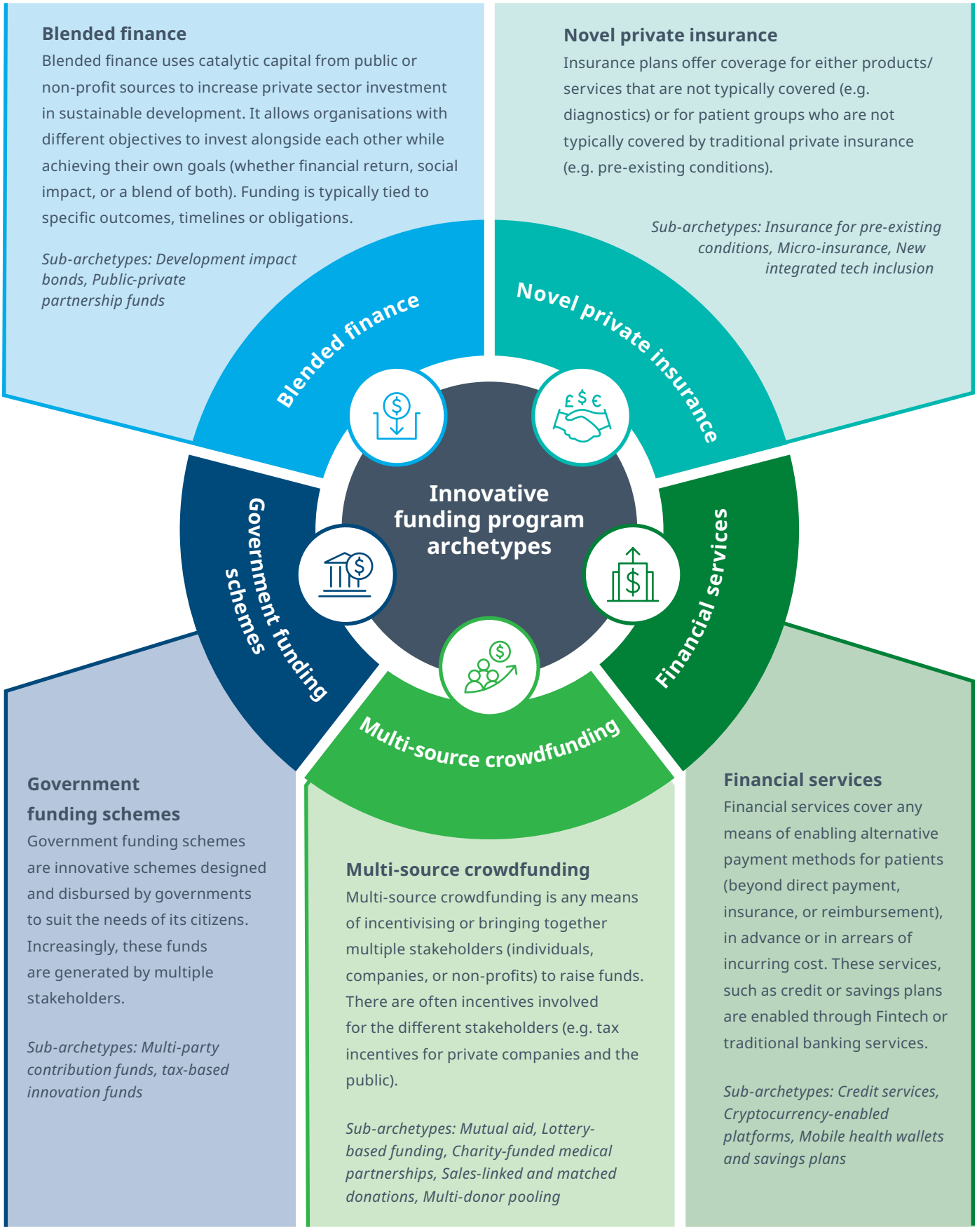


Figure 5. 5 archetypes of innovative funding

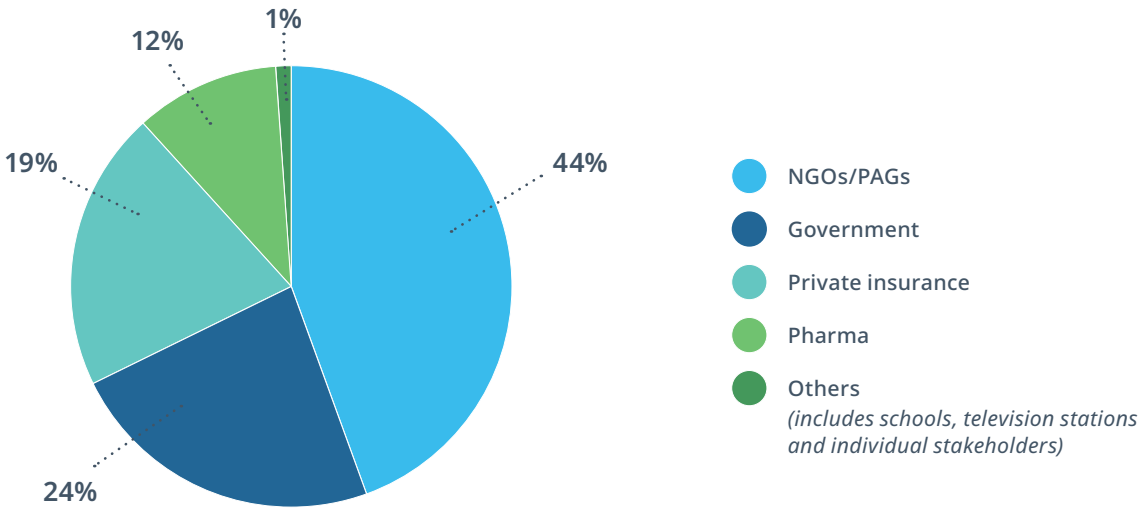


The majority of the identified rare disease programs are focused on fundraising archetypes, patient access programs and donations as the funding framework for rare disease is still in development across the globe. Only ~20% of identified funding programs can be considered innovative (e.g. government intervention), leaving an opportunity for innovative funding programs to unlock new sources of funds to deploy benefits to patients in need of funding.

Innovation in funding can come from creating a new source of funds or pools, a collaboration of non-traditional stakeholders to form a new mechanism, or the leverage of technological developments to address a funding challenge.

Additionally, we looked at who were the primary stakeholders for the implementation of funding models. Figure 6 shows that Non-government Organisations (NGOs)/Patient advocacy groups (PAGs) have been the most important stakeholder in launching funding models for Rare Diseases. This result is expected given the high involvement and history these organisations have with regard to fundraising and donation campaigns. Moreover, the government and pharmaceutical companies both play essential roles in designing and implementing this funding mechanism. This result may lead to multi-stakeholder engagements in the future where funding programs could be co-created among the different parties interested in strengthening patient access to RD.

Figure 6. Key implementation stakeholders of funding programs



Major stakeholders	Major players	Major funding program archetype
NGOs/PAGs	Regional/global organisations: • Global Genes • SMA Foundation • Cure SMA	• Fundraising • Donation
Goverment	• Ministry of Health • Ministry of Public Health	• Govt. interventions • PAPs • Reimbursement agreements
Pharmaceutical companies	• Biogen • Sanofi • Novartis	• PAPs

Rare disease case studies

The selected case studies in this section highlight innovations in pooling or mobilisation of resources to finance treatment costs for patients and future patients in rare diseases. They have been selected to showcase the possibilities of innovative solutions in tackling funding gaps in the countries.

Government funding schemes[6-17]

These are programs that aim to provide financial coverage through social provisions and national collaborations. “Special” funds are important to fund sources for securing medication for people with rare diseases. These are programs that aim to provide financial coverage through social provisions and national collaborations. Special funds can be roughly divided into private charitable funds, local special funds, and special national funds. Local special funds are usually led by local governments or institutions with a government background and are intended to be used as additional support beyond local medical insurance. National government departments or national institutions establish national special funds, and usually have many project sites throughout the country, with broad coverage.

Government funding schemes *National special funds*

Singapore

The Singapore Rare Disease Fund (RDF)

A public-private partnership to provide long-term financial support for patients with rare diseases requiring treatment with high-cost medicines.

Program design & offering

- For every S$1 of public donation, the government will triple the amount, boosting the impact of the fund
- Tax deduction of 250% are provided for eligible donations
- RDF donor wall is published on KKH’s website to recognise the fund donors

Key stakeholders

- Ministry of Health, Singapore
- NGOs (i.e. Community Chest Singapore)
- National Fund Committees (i.e. SingHealth)
- Private companies (e.g. private insurance providers)
- General public

Outcomes

~USD 70m have been raised to date to support children with these diseases:

- Primary bile acid synthesis disorder
- Gaucher disease
- Hyperphenylalaninemia
- Pompe disease

Government funding schemes *Comprehensive support system*

Taiwan

Taiwan's comprehensive support system for rare diseases

Since 2000, Taiwan has established an efficient system to protect and support patients with rare diseases by providing: legal disease, social and financial support.

Program design & offering

- Taiwan enacted the Rare Disease Control and Orphan Drug Act (2002) and introduced the Rare Diseases and Rare Genetic Disorders Care and Services Plan (2007)
- The policies set forth the rare diseases definition and established a review committee, focused on registration, prevention, diagnosis and treatment of rare diseases, patient and social care, legal protection and financial support

Key stakeholders

- Ministry of Health and Welfare, Taiwan
- Rare Diseases and Medicine Review Committee
- Patient organisations (i.e. Taiwan Foundation for Rare Disorders)
- Local health insurance entity

Outcomes

- Established a well-functioning support system for rare diseases
- Within the first 4 years, over 140 kinds of rare diseases were covered by the National Health Insurance (NHI), reducing patient economic burden
- The NHI has a separate fund that utilizes 24.2% of the tobacco tax to pay medical expenses for rare diseases and sustainable financial support

Government funding schemes *National special funds*

Romania

Romanian rare disease ring-fenced funds

A ring-fenced fund was created to provide equitable and sustainable access to RD and orphan drug treatments and care to patients living with a rare disease in Romania.

Program design & offering

- A dedicated budget under the federal government funds is currently allocated to cover at least one treatment for 27 listed rare diseases
- From 2014, the National Plan for Rare Diseases was adopted to recognise rare diseases as a priority in Romania
- The National Program for Treatment of Rare Diseases is funded through the legislation approving the implementation methodology of the National Public Health Programs

Key stakeholders

- Ministry of Health, Romania
- Romanian National Alliance for Rare Diseases
- National Council for Rare Diseases

Outcomes

- Secured designated share of RD coverage and funding for treatment to the population in Romania
- In 2021, the budget allocated specifically for the National Program for Treatment of Rare Diseases will be over USD 90 million

Government funding schemes *National special funds*

Italy

Italian rare disease ring-fenced funds

Italy has allocated special funds for innovative drugs with differing types of public funding pathways based on the status of market authorisation to bring more patient access for rare disease therapies.

Program design & offering

- Italy's National Health Service has full public coverage, funded primarily through corporate and value-added tax revenues
- AIFA 5% fund: National law allowing physicians to request funding for orphan drugs before market authorisation, contributed by companies (5% of promotional expenses)
- Innovative drug fund: Funding for orphan drugs which achieved full innovative designations

Key stakeholders

- National Health Service, Italy
- Italian Medicines Agency (AIFA)
- Corporate healthcare companies

Outcomes

- AIFA 5% fund is used to reimburse patient's cost of treatment, promote orphan disease research and improve access to drugs awaiting market entry
- In 2017, ~65% of the fund applications (82 patients) have benefited with an expenditure totaling €14 million
- Enables a sustainable funding pathway for SMA patients for approved therapies including Spinraza

Government funding schemes *Reimbursement agreements*

Italy

Zolgensma innovative payment model

In Italy, Zolgensma engaged an innovative payment model, featuring different access agreements, which offers reimbursement benefits and a better life prospect for children with SMA.

Program design & offering

- Key stakeholder negotiation and commitment between government and pharmaceutical companies
- Gene therapy Zolgensma has been included in Italy's innovative drug fund (funding for orphan drugs which achieved full innovative designations)
- To support pediatric access, patient weight was used as the negotiating factor for different agreements
  - » Under 13.5kg: payment-at-results contract
  - » Between 13.5 to 21kg: product is free of charge through a clinical trial program

Key stakeholders

- National Health Service, Italy
- Italian Medicines Agency (AIFA)
- Pharmaceutical and biotech companies (i.e. Novartis)

Outcomes

- Agreements based on weight category allowed different patient groups to access treatment at a sustainable price, including obligatory discounts
- Free clinical trial provision of Zolgensma enabled acquiring of additional efficacy and safety data on heavier patients

Novel private insurance

Pre-existing diseases such as hereditary, genetic conditions are typically excluded from private insurance coverage and therefore, private insurance for rare diseases is rarely available. Insurers have a limited understanding of rare diseases and see little demand for coverage. However, some insurers are starting to see the opportunity of offering disruptive, innovative solutions for new areas with high unmet needs.

The following programs, under the novel private insurance archetype, offer comprehensive or complementary coverage for products, services or patient groups that would not typically be covered by traditional private insurance. Novel private insurance solutions can leverage a variety of channels, including mobile and retail technology, enabled by collaboration between key public-private stakeholders.

Novel private insurance | **China**

ZhongAn Rare Disease Insurance

InsurTech company ZhongAn offers easier access, wider coverage and more payment options for quality medical care of 121 rare diseases.

Program design & offering

- ZhongAn is an online insurer providing coverage across lifestyle consumption, consumer finance, health, auto and travel, in the Chinese market
- Ensuring collaboration and compliance among private insurers and government
- Key Million Medical individual insurance product, Zunxiang 2020, extends the coverage to 121 types of rare diseases including Haemophilia

Key stakeholders

- Ministry of Industry and Information Technology, China
- Local health insurance start-ups and companies

Outcomes

- Provides personalised financial coverage for population of specific rare diseases, through discounted drugs and cure effect coverage
- If diagnosed with specific rare diseases such as Haemophilia after enrolment, the medical expenses generated will be covered

Supplementary insurance

China

City Supplementary Commercial Health Insurance (CSCHI)

A localised supplemental plan that leverages on strong government support and few enrollment restrictions to provide an inclusive and more affordable health insurance for the city's residents.

Program design & offering

- CSCHI is supplementary to the basic medical insurance provided by the Chinese government
- Over 120 CSCHI products are launched so far, mainly focused on the coverage of serious illnesses
- Rare diseases are mostly covered within the scope of inpatient medical costs, with no drug limitation as long as products are prescribed in local public hospitals

Key stakeholders

- China Banking and Insurance Regulatory Commission
- Local government
- Local healthcare insurance entity (i.e. Ping An)

Outcomes

- In Foshan, Guangdong, inpatient medical costs can be insured up to CNY 1 million at a competitive price
- Ease the burden of extra medical expenses which are not covered by the national medical insurance plan
- Easier and wider access to drugs and treatment for varied local patient groups (e.g. older people, people with pre-existing conditions)

Expert inputs on implementation

Experts consulted as part of the LMIC assessment, which covered the **Funding Environment for Rare Diseases in Low and Middle Income Countries report**, provided insights relevant for global programs. They proposed that five strategic areas need to be addressed in the implementation of an innovative funding program involving a multi-stakeholder ecosystem:

1. **Disease awareness:** Awareness should focus beyond the financial aspect of the disease (e.g. including the psychosocial part of the disease)

2. **Patient perspective:** Include patients' perspectives to develop an impactful message and to align key stakeholders (e.g. payers) on incentives or opportunities

3. Countries to modify reimbursement decisions to improve access to orphan drugs:

    - **Adopt a multidisciplinary HTA framework** that is evidence-based and not focused entirely on cost-effectiveness

    - **Adoption of specific payment mechanisms** to accommodate high uncertainty of orphan drug effectiveness and for data generation such as risk-sharing agreement

4. **Payer and policymaker engagement:** Communication of unified voice together with supportive data (e.g. rare disease financial and psychosocial impact study) to form an open conversation on sustainable funding solutions

5. **Sustainable solutions:** Ensure solutions formed are based on continuous and sustainable efforts (e.g. partnerships that meet collective and individual stakeholder interests)

Conclusion

In most countries, across different rare diseases, there are common access challenges for patients to access much-needed treatments, one of which is a lack of sustainable funding. Through a combination of desktop research and interviews with local stakeholders, this study collected information on over 130 funding-related initiatives across 40 different nations, constituting to our knowledge one of the largest compilations of its kind. This collection presents many valuable insights on the characteristics of available funding mechanisms for RD and, at the same time, can be interpreted as a natural response to the funding gap that currently exists in the space of rare diseases.

This study discovered a wide range of funding models which, when complementing traditional funding, can have a big impact on those who do not currently have access to appropriate care for rare diseases due to affordability. However, unlike more prevalent NCDs (e.g. oncology), only ~20% of identified funding programs can be considered innovative, leaving an opportunity for innovative funding programs to unlock new sources of funds to deploy benefits to patients in need of funding and indicating that there is much more to be done in this space.

Since these programs alone will be insufficient to address the growing RD burden across the world, a strategic approach focused on the co-creation among stakeholders of innovative funding models that complement long-term government initiatives, such as the attainment of uninversal healthcare (UHC), should be implemented to ensure sustainability.

Partnerships will be critical to creating holistic programs focused on more than one rare disease area. Patient groups, providers, NGOs, foundations, physician communities are key influencers for policymaking and can be potential partners for funding and implementation.

The challenge for policymakers and rare disease stakeholders such as patient organisations and the private sector will be to identify funding solutions most appropriate for that health system and the population's needs and how to sustainably adopt them. It is also a priority to build adequate evidence for rare disease funding prioritisation and accompany it with strategic guidance on approaching funding and financing solutions. In addition, it will be crucial to focus on identifying best practices and key learnings of similar international experiences and identifying external experts that can support discussions with key decision makers to create the required innovative solutions.

Partnerships will be critical to creating holistic and comprehensive programs, which can be focused on more than one rare disease area. Patient groups, providers, NGOs, foundations, biotech companies, and physician communities are key influencers for policymaking and can be potential partners for funding and implementation. Partnering will also play a key role in gathering and presenting robust evidence on the burden of RD and the impact of the funding gap to mobilise actions towards appropriate care of RD patients and families.

"Partnerships will be critical to creating holistic and comprehensive programs"

Acknowledgements

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References

1. Web article. “EU research on rare diseases”. European commission. Last accessed on April 17th 2020. https://ec.europa.eu/info/research-and-innovation/research-area/health-research-and-innovation/rare-diseases_en
2. Web article. “FAQs About Rare Diseases”. NIH. Last accessed on April 17 2020. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases
3. Web article. “Rare diseases”. Findacure. Last accessed on April 17 2020. https://www.findacure.org.uk/rare-diseases/
4. Web article. “Cancer”. Our World in Data. Last accessed on April 17 2020. https://ourworldindata.org/cancer
5. Atun Rifat, Silva Sachin et al. Innovative financing instruments for global health 2002–15: a systematic analysis. The Lancet, Volume 5, Issue 7. Published July 2017. https://www.thelancet.com/journals/langlo/article/PIIS2214-109X(17)30198-5/fulltext
6. “Rare Disease and Orphan Drug Act--Achievement Summary”. Health Promotion Administration. Last modified on January 29 2015. https://www.hpa.gov.tw/EngPages/Detail.aspx?nodeid=1075&pid=6178
7. “Prevention and Healthcare of Rare Diseases”. Health Promotion Administration. Last modified on March 29 2019. https://www.hpa.gov.tw/EngPages/Detail.aspx?nodeid=1075&pid=10528
8. Jason C. Hsu et al. Disease and economic burden for rare diseases in Taiwan: A longitudinal study using Taiwan’s National Health Insurance Research Database. Published September 2018. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6150502/

9. “Suffering in silence: Assessing rare disease awareness and management in Taiwan”. CSL Behring and The Economist Intelligence Unit. July 2020. https://www.cslbehring.com/-/media/shared/economist-white-paper/eng_snapshot_a4_digital-taiwan-final.pdf

10. “Applications made for rare disease fund”. Ministry of Health Singapore. February 3 2021. https://www.moh.gov.sg/news-highlights/details/applications-made-for-rare-disease-fund

11. “Applications for rare disease fund”. Ministry of Health Singapore. April 5 2021. https://www.moh.gov.sg/news-highlights/details/applications-for-rare-disease-fund

12. “Update on rare disease fund progress”. Ministry of Health Singapore. November 3 2020. Last accessed on 16 July 2021 https://www.moh.gov.sg/news-highlights/details/update-on-rare-disease-fund-progress

13. National plan for rare diseases: Romania. Europlan 2017 Romania National Conference Report. Last accessed on 16 July 2021 http://download2.eurordis.org/rdpolicy/National%20Plans/Romania/4.EUROPLAN_2017_Romania%20National%20Conference_Report_English.pdf

14. MoH Gazzetta Ufficiale n. 274 AIFA Innovative Drug Fund. Last accessed on 16 July 2021 https://www.aifa.gov.it/documents/20142/241044/fondo_AIFA5.pdf/afe55ea9-9d38-76c4-bade-690d8050ef05

15. Gazetta Ufficiale della Repubblica Italian - Reimbursement of Zolgensma. Last accessed on 16 July 2021 https://www.gazzettaufficiale.it/atto/serie_generale/caricaDettaglioAtto/originario?atto.dataPubblicazioneGazzetta=2021-03-13&atto.codiceRedazionale=21A01554

16. “Italy Reimburses Zolgensma Through “Payment at Result” Model, Priced at EUR 2.155 M”, Eversana. Last accessed on 16 July 2021 https://www.eversana.com/2021/03/18/italy-reimburses-zolgensma-through-payment-at-result-model-priced-at-eur-2-155-m/

17. “AIFA approves reimbursement of Zolgensma, gene therapy for children with SMA1”, AIFA. Last accessed on 16 July 2021 https://www.aifa.gov.it/en/-/aifa-approva-rimborsabilita-zolgensma-terapia-genica-per-i-bambini-con-sma1

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Will is responsible for leading regional and global strategy consulting projects at IQVIA Asia Pacific, based in Kuala Lumpur, Malaysia. In his current role, Will specializes in market access and global health systems, advising industry players, governments, and global health agencies. Starting his career in the Europe and continuing in Southeast Asia, he has gained a wealth of experience across both developed and emerging healthcare markets globally. He holds a MEng in Chemical Engineering from Imperial College London, UK.

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With regional headquarters in Singapore and offices in 15 countries, IQVIA Asia Pacific provides technology-enabled services and solutions to meet the growing and rapidly changing needs of clients, both local and multinational, operating in Asia Pacific. IQVIA is committed to advancing healthcare by offering evidence-based insights and deep domain expertise in thought leadership, with the aim of improving understanding and accelerating innovation within the healthcare ecosystem.

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