Unlocking access to medicines for rare diseases in Southeast Asia

Approval number: C-ANPROM/GEM/CORP/0068.

Date of preparation: July 2024

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Abbreviations

Abbreviation	Term
AADC	Aromatic L-amino acid decarboxylase
EB	Epidermolysis bullosa
HAE	Hereditary angioedema
HSS	Healthcare systems strengthening
NF-1	Neurofibromatosis type 1
NHI	National Health Insurance
NHSO	National Health Security Office
NLEM	National List of Essential Medicines
PAG	Patient advocacy group
PCP	Primary care physician
RDF	Rare Disease Fund
UCS	Universal Coverage Scheme
XLH	X-linked hypophosphatemia

1. Introduction

The Southeast Asia Rare Disease Summit 2024 convened over 600 participants to discuss patient empowerment and policy changes as tools to improve diagnosis and access to medicines for patients with rare diseases. The summit brought together healthcare professionals and policy makers, creating an opportunity to share expertise and experience, foster collaboration, and drive real change for patients with rare diseases in Southeast Asia. Patient voices were central to the summit, with representatives from rare disease patient advocacy groups (PAGs) in Indonesia, Malaysia, the Philippines, Singapore, and Thailand showcasing initiatives currently under way to improve access to medicines for patients with rare diseases in their countries, as well as outlining plans for the future.

This white paper presents the opportunities for overcoming barriers faced by patients with rare diseases by demonstrating the progress made by individual countries in the region. In keeping with Takeda's commitment to healthcare systems strengthening (HSS) [box 1], we describe locally led initiatives integrated with existing healthcare systems and infrastructure in Southeast Asia to ensure sustainable improvements in access to medicines for people living in the region. We also share progress on activities around the patient journey and advocacy, highlighting success factors that could influence implementation in other regions.

Box 1

Takeda's approach to HSS is fundamental to its vision of accelerating patient access to medicines. HSS initiatives seek to reinforce the capacity of a healthcare system in delivering care through robust programs with impact measures incorporated from the outset, focusing on gaps in these core focus areas:

- **The patient journey**, including all steps from disease awareness to screening, diagnosis, treatment, and aftercare
- **Health education**, for both patients and healthcare workers
- **The supply chain**, ensuring the uninterrupted availability of medicines
- **Advocacy**, shaping policy to improve access to healthcare and medicines for patients

2. The burden of rare diseases in Southeast Asia

Around 6000 known rare diseases are currently recognized by international researchers, affecting up to 1 in 17 people worldwide and 45 million people across Southeast Asia.[1,2] Patients with a rare disease face challenges throughout the patient journey, from a lack of disease awareness to treatment. Missed diagnosis and misdiagnosis are extremely common, and patients who do have a diagnosis are unlikely to receive effective treatments.[3] Targeted treatments have only been developed for approximately 5% of rare diseases, and where they are available, patients face logistical and affordability barriers to access.[3] As such, most patients with a rare disease only receive symptomatic treatments, resulting in reduced quality of life and poor social and economic outcomes.[4,5] Furthermore, they are at risk of early death if they are not diagnosed and treated in a timely manner.[6] Families are similarly affected, often becoming overwhelmed by caregiving duties.[7]

Despite the extensive challenges faced by people living with a rare disease, it remains an overlooked area as few people are affected by each disease compared with the large numbers of people affected by more common diseases.[8] In particular, as discussed during the summit, there is a perception that developing rare disease treatments and working to improve access to them will have a low return on investment for research institutes, governments, and pharmaceutical companies, which would rather focus their efforts on addressing more common health concerns.

3. Unlocking access to medicines for rare diseases in Southeast Asia

Patients with a rare disease across Southeast Asia face unique and varied challenges owing to the different governments, health systems, and patient attitudes in the region. During the Rare Disease Summit, representatives from Southeast Asian nations discussed the strengths and weaknesses of the rare disease treatment continuum in their countries and suggested that Southeast Asian countries look to one another for positive examples of how to implement policies that truly benefit patients with a rare disease.

In this section, we examine the barriers to rare disease diagnosis and care in Southeast Asia and demonstrate how these barriers have been overcome in individual countries through case studies; finally, we demonstrate the rationale for each case study, which may guide other Southeast Asian nations in implementing similar solutions.

3.1 Patient journey

3.1.1 Newborn screening in rare diseases

Barriers:

For patients with a rare disease, the length of time between birth and diagnosis is critical; if treatments are available, it is often recommended that patients receive these as early in life as possible to maximize benefits.[9] Some countries implement comprehensive newborn screening programs for rare diseases to improve the rates of early diagnosis.

Case study:

Newborn screening in Singapore
All children born in Singapore hospitals are routinely offered newborn screening [box 2],[10] with funding for screening supplied through the national insurance system in Singapore, known as MediSave. Upon registration of a child's birth, the government credits 4000 SGD to the child's MediSave account, which can subsequently be used to defray the costs of healthcare expenses.[11]

The program is supported by the purpose-built National Expanded Newborn Screening Laboratory. This laboratory screens 90% of samples from an average of 40,000 children born in Singapore each year.[10] The implementation of this screening program in Singapore has improved the rate of early diagnosis of rare diseases and led to the timely initiation of treatment in presymptomatic patients.

Box 2

Conditions that are routinely screened at birth in Singapore: [10]

- Inborn errors of metabolism
- Cystic fibrosis
- Congenital adrenal hyperplasia
- Severe combined immunodeficiency
- Galactosemia
- Biotinase deficiency
- Hearing abnormalities
- Glucose 6-phosphate deficiency
- Thyroid function

Recommended call to action

Implement newborn screening programs that are adequately supported by funding and genetic testing services

Rationale: Implementing a successful newborn screening program depends on the availability of funding and genetic testing services. It is important to note that in some Southeast Asian countries, these barriers are compounded by geographic isolation, such as in the Philippines, an archipelago of more than 7000 islands.[12] According to representatives at the Rare Disease Summit, Filipinos rely on a limited network of seven genetic testing laboratories and 33 newborn screening continuity clinics to cover this large area. Combined, these clinics are responsible for implementing newborn screening and follow-up care for a population of more than 111 million.[12]

3.1.2 Later-in-life diagnosis

Barriers:

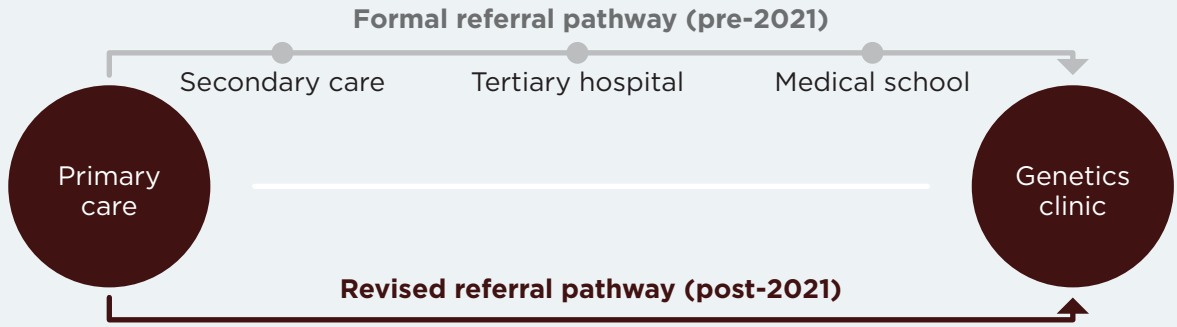
Primary care physicians (PCPs) are generally unfamiliar with the myriad of symptoms associated with rare diseases because of the large number of such diseases and low levels of awareness.[13] Furthermore, there is a lack of specialists familiar with rare diseases.[14] At the Rare Disease Summit, representatives from multiple Southeast Asian countries shared that owing to this lack of knowledge, centralized referral is common, meaning that patients often have to travel long distances to see a specialist and obtain an accurate diagnosis.

Case study:

Simplification of referral pathways in Thailand

During the Rare Disease Summit, presenters from Thailand shared an update on the simplification of referral pathways for patients with a suspected rare disease. Such patients are primarily served by seven genetics clinics distributed around the country. Prior to 2021, access to these clinics was under heavy gatekeeping, with patients requiring at least four referrals to see a geneticist. The referral system was simplified in 2021 to support an expansion in benefits for patients with rare diseases [box 3]. Now, patients only require a single referral from a PCP to see a geneticist.

Box 3



Recommended call to action

Educate PCPs and simplify referral pathways to facilitate timely, accurate diagnosis

Rationale: Owing to the large number of rare diseases, educating PCPs on the signs and symptoms of each presents challenges; however, PCPs should be trained to recognize the broad signs and symptoms of rare diseases through targeted education. Subsequently, the simplification of referral pathways is important to reduce the time to diagnosis and treatment. Referral protocols that promote the referral of patients from PCPs directly to specialists are needed to reduce the amount of time spent in primary care without a diagnosis.[14]

3.1.3 Emergency and acute care

Barriers:

Patients with a rare disease often experience urgent or acute health problems that need to be addressed by a specialist in a timely manner. However, physicians in emergency departments are often unfamiliar with rare diseases and their management,[15,16] meaning that patients may receive inadequate or inappropriate care because of this lack of specialist knowledge.

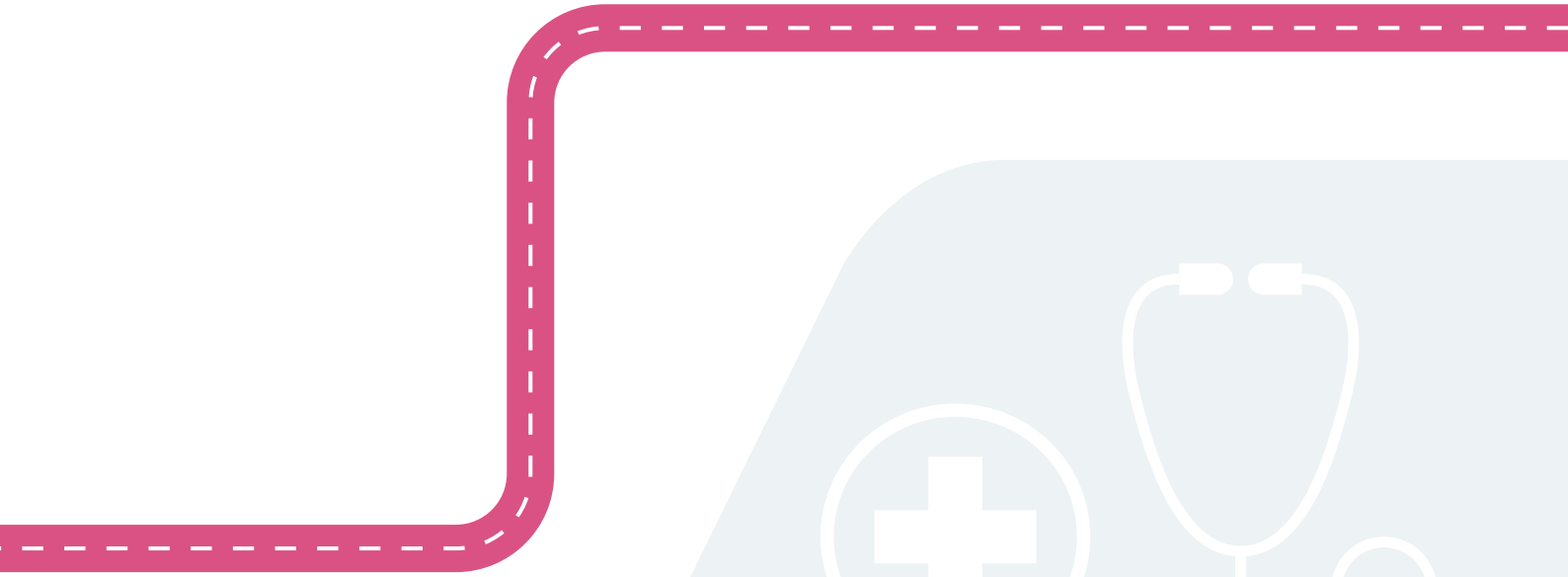
Case study:

During the Rare Disease Summit, representatives from the Rare Disorders Society of Singapore discussed plans to distribute 'rare disease cards' to its members in the near future. These cards will provide details of the patient's rare disease and act as a communication tool for those being admitted for emergency care. In 2014, a similar initiative was planned and executed by the French government in response to similar concerns surrounding the treatment of patients with a rare disease in emergency departments.[16]

Recommended call to action

Collaborate with PAGs for simple, effective solutions to real, everyday barriers to patient access

Rationale: Simple steps can be taken to improve access to appropriate care for people living with a rare disease. PAGs play a key role in identifying the needs of people living with a rare disease. Collaboration between PAGs is needed to ensure that patients and caregivers can access broad and disease-specific resources, and to connect families with similar rare diseases.



3.2 Advocacy

3.2.1 Development and coverage of innovative treatments

Barriers:

Targeted treatments, which generally provide better outcomes than symptomatic treatments, are available for approximately 5% of rare diseases.[3] Further research and funding are needed to develop treatments for more rare diseases.

Among patients who are eligible for targeted therapies, regulatory and affordability barriers prevent access to these life-changing treatments. In Southeast Asia, regulatory and reimbursement processes are lengthy and often fragmented[17–19] leading to delays in patients accessing treatments that may already be available in Europe or the USA. Targeted treatments are often expensive, so, once approved, they are unlikely to be reimbursable under public healthcare policies in many Southeast Asian nations.[2] Discussants at the Rare Disease Summit stated that import and export regulations also present significant barriers to access for reimbursable treatments, or treatments that patients attempt to acquire with private funds.

Proper implementation of policies to protect the rights of people living with rare diseases, to improve the timeline for registration and reimbursement of targeted treatments, and to provide adequate funding are needed to improve outcomes for those living with a rare disease.

Case study 1:

Development of the Orphan Drugs and Rare Diseases Act of Taiwan
In 1999, the Taiwan Foundation of Rare Diseases was founded and subsequently campaigned for the introduction of an Orphan Drugs and Rare Diseases Act. The Act, which was passed in the year 2000, specifies the following:[20]

- A rare disease is defined as any disease with a prevalence of <1 in 10,000 in Taiwan
- Patients with a rare disease are exempt from National Health Insurance (NHI) co-payments related to rare disease treatment
- Subsidies for medical equipment and costs not reimbursable under the NHI, such as special dietary requirements, shall be made available to patients with a rare disease
- The application fee for marketing approval of orphan drugs shall be reduced to a nominal fee of 341 USD (vs 51,181 USD for non-orphan drugs)
- Sponsors may apply for priority review and accelerated approval of orphan drugs based on surrogate endpoints
- Central authorities shall provide funding support to encourage research related to the prevention and control of rare diseases

Amendments to the Act in 2009 designated a specific proportion of taxes from alcohol and tobacco sales to support access to treatments for rare diseases, ensuring that a designated fund is available on an ongoing basis.[20]

Recommended call to action

Collaborate with PAGs to develop and implement relevant supporting legislation that considers multiple aspects of the rare disease treatment paradigm

Rationale: Following the example of the Orphan Drugs and Rare Diseases Act of Taiwan, it is vital to define what a rare disease is in order to develop relevant supporting legislation;

only with a definition can adequate care be provided to those in need. This Act identifies and designates an ongoing source of funds,[20] which is essential to support patients with a rare disease. As shared by representatives from PAGs during the Rare Disease Summit, Rare Disease Acts should not only consider improving the affordability of care for patients with rare diseases; the streamlining of regulatory pathways and provision of funds to support research into rare diseases are also important. PAGs have a strong voice and, in this case, were able to lobby the government for the development of a Rare Disease Act.

Case study 2:

Integration of care and medication reimbursement for rare diseases in Thailand
In Thailand, healthcare is provided to the majority of the population through the Universal Coverage Scheme (UCS), with UCS packages (comprising entitlement to diagnostic services, right to specialist visits, etc) determined by the National Health Security Office (NHSO). For a medication to be approved for reimbursement through the UCS, it must also be listed on the National List of Essential Medicines (NLEM), with applications for inclusion in the NLEM handled by a dedicated subcommittee for NLEM development.[21] This two-part system complicates rare disease management as there is a need to include both diagnosis and treatment under the UCS.

During the Rare Disease Summit, representatives from Thailand discussed the establishment of the Rare Disease Working Group in 2019. This group assumed responsibility for improving access to treatments for patients with rare diseases and was subsequently formalized as the Rare Disease Development Working Group in 2023, with the aim of developing a comprehensive care system for patients with rare diseases. This working group is now responsible for the selection and review of rare diseases for inclusion under the UCS and coordinates timely UCS and NLEM applications, ensuring comprehensive access to healthcare. As a result of these changes, the working group has achieved:

- The inclusion of expanded newborn screening and coverage for 24 inborn errors of metabolism under the UCS benefits package
- The identification of six further rare diseases for UCS benefits package development

Recommended call to action

Include access to both diagnosis and treatment of rare diseases as part of universal healthcare coverage

Rationale: Patients with a rare disease require integrated diagnostic and medical services. As such, reimbursement for diagnostic and medical services should be considered at the same time. As demonstrated in Thailand, developing a designated working group to prioritize the review of benefits for people with rare diseases could speed up the process of establishing reimbursement for essential treatments.

Case study 3:

Indonesia's Special Access Scheme for medications to treat rare diseases
Since 2017, Indonesians who import medicines for the treatment of rare diseases from abroad for personal use may do so via the Special Access Scheme. Medicines imported under this scheme are tax exempt and will be immediately released from customs control upon receipt of the permit.[22] During the Rare Disease Summit, a spokesperson for the Indonesian Rare Diseases Foundation (Yayasan MPS Dan Penyakit Langka Indonesia) outlined how the society has collaborated with the Ministry of Health, the Ministry of Finance, the Indonesian FDA, and the National Pharmacy Company to develop a memorandum of understanding for this scheme; the spokesperson also highlighted the importance of multistakeholder collaboration and PAG involvement in shaping rare disease policy.

Recommended call to action

Develop streamlined import and export schemes for rare disease medications

Rationale: As shared by representatives of PAGs during the Rare Disease Summit, patients with rare diseases in Southeast Asia often need to import medications from abroad. The streamlining of import and export schemes ensures efficient access to medications where needed and prevents a backlog of medications accumulating at customs checkpoints. This may be particularly important in acute or emergency situations in which rapid access to appropriate medications could make a difference in patient outcomes.

Case study 4:

Public–private partnership to raise funds for rare disease treatments in Singapore
In Singapore, the Rare Disease Fund (RDF) is a charity created to fund access to high-cost medicines for patients with rare diseases. Established in collaboration with the Ministry of Health and the SingHealth Fund, any donations made to the RDF are matched 3:1 by the government, with the government also providing all funds for the maintenance of the charity.[23,24]

Recommended call to action

Scale up public–private partnerships to streamline RDF/charities' navigation of all available funding support (including government, not for profit, charitable, and private/corporate)

Rationale: As demonstrated in Singapore, charities may play an important role in funding treatments for rare diseases in Southeast Asia. Government buy-in increases sustainability and, combined with public–private partnerships, can magnify the efforts of charities, thus increasing the benefits for people with rare diseases.

3.2.2 Empowering patients to drive change: PAGs and support groups

Barriers:

Patients with rare diseases and their families play a pivotal role in advocating for increased quality of care and access to medicines.[25] Many patients perform extensive research and become experts in their own disease,[26] representing valuable sources of knowledge for policy makers and healthcare professionals. Conversely, people newly diagnosed with a rare disease and their caregivers often require psychosocial support beyond that which can be offered by governments and healthcare providers.[25] During the Rare Disease Summit, representatives of PAGs opined that owing to the small number of patients with each rare disease and the geographic spread of many countries in Southeast Asia, the voice of PAGs could be diluted. In recent years, disease-specific PAGs have banded together, creating rare disease societies and foundations that work together to raise the voices of all patients living with rare diseases in Southeast Asia. During the summit, PAG members identified that while consolidated PAGs are important to push a broad agenda for patients with rare diseases, they should also support autonomy for disease-specific PAGs, which are essential in providing psychosocial support for patients and tackling disease-specific challenges.

Case study 1:

Disease-agnostic PAGs in Southeast Asia joining together to raise the voices of people with rare diseases

1. The Thai Rare Disease Foundation provides support to patients living with all types of rare diseases in Thailand and aims to bring together patients with similar conditions to exchange information and experiences. Besides directly helping patients, the foundation is involved in the support and development of disease-specific PAGs in Thailand [box 4]. Furthermore, members of the society sit in committees consulting on the inclusion of rare disease benefits under the UCS, as well as medications for the treatment of rare diseases on the NLEM.[27]

Box 4

Disease-specific PAGs supported by the Thai Rare Disease Foundation[27]

- Achondroplasia
- X-linked hypophosphatemia (XLH)
- Neurofibromatosis type 1 (NF-1)
- Epidermolysis bullosa (EB)
- Aromatic L-amino acid decarboxylase (AADC) deficiency
- Hereditary angioedema (HAE)
- Lysosomal storage disorders
- Rett syndrome
- Angelman syndrome

2. The Rare Disorders Society Singapore exists to improve the lives of patients living with rare diseases, embracing equity and inclusivity to empower patients. The society provides information about rare diseases, as well as psychosocial and financial support to more than 200 patients with rare diseases and their families.[28] The society holds an annual race and carnival known as 'Carry Hope', which attracts sponsorship from pharmaceutical companies and international private entities. In 2023, over 1000 attendees joined the event, including the Minister for Health, Mr Ong Ye Kung, helping to raise awareness of rare diseases.[29] During the Rare Disease Summit,a representative of the society, shared the opinion that gaining support for rare diseases in Singapore depends on tapping into the national political agenda, which may be more concerned about aspects often considered secondary to patients with rare diseases, such as the economic activity of caregivers. As mentioned at the summit, the society would like to launch community-based support centers and establish a Singapore Rare Disease Registry in the next 5 years.

3. The Malaysian Rare Disorders Society is a voluntary organization representing the welfare of individuals affected by rare diseases and their families. The society's key aims are to maintain a strong rare disease network in Malaysia, raise community awareness about rare diseases, provide psychosocial support to individuals and families affected, empower youth affected by rare diseases, and support the establishment of policies and acts benefiting people with rare diseases. The society has published a children's book about rare diseases and supported the publication of a manuscript on the diagnosis of rare diseases among adults. The society is also a key voice in the development of rare disease policy in Malaysia, with representatives sitting in the National Committee for Rare Disease and thereby influencing the national rare disease budget.[30]

4. The Philippine Society for Orphan Disorders aims to improve the welfare of individuals with rare disorders by directly addressing, supporting, and protecting their health and general well-being. The charity collaborates with healthcare providers and local government units to arrange transport for patients in geographically isolated areas, ensuring that they can access facilities run by the Center for Human Genetic Services for comprehensive clinical evaluation, appropriate management, and genetic counseling.[31]

5. As discussed during the Rare Disease Summit, the Indonesian Rare Diseases Foundation advocates for the rights of people living with rare diseases in Indonesia and provides opportunities for meaningful connection and support between patients. During the Rare Disease Summit a spokesperson for the foundation outlined how diagnostics are a key focus: the foundation has established an international partnership with National Taiwan University Hospital to enhance the provision of diagnostics for patients with suspected lysosomal disorders and has been an influential voice in discussions surrounding the establishment of a human genetic research center at the University of Indonesia.

During the Rare Disease Summit, representatives from the above PAGs announced that in the near future, these groups plan to join together to form the ASEAN Rare Disease Consortium, with the aim of improving cross-border collaboration, developing regional best practices, and further strengthening the voices of patients with rare diseases in Southeast Asia.

Recommended call to action

Consolidate PAG efforts for a louder patient voice

Rationale: During the Rare Disease Summit, representatives from PAGs discussed how patients with different types of rare diseases share similar needs and stated that the development of rare disease societies, which oversee or combine many PAGs, amplifies the voices of patients with rare diseases. Despite the formation of larger groups, individual rare disease groups remain important for psychosocial support. PAGs should collaborate with multiple stakeholders, including governments and private entities, to raise awareness and funds and implement policy changes.

Case study 2:

Structuring consolidated PAGs to provide support to disease-specific PAGs

The Malaysian Rare Disorders Society has brought together more than 320 individuals with over 100 rare diseases.[30] As shared by representatives of the Society during the Rare Disease Summit, and with the aim of acknowledging the breadth of experiences of people living with rare diseases, the group maintains disease-specific networks throughout the country. Individuals with specific rare diseases are connected by the Malaysian Rare Disorders Society and subsequently provided with support and guidance to form and maintain their own PAGs, with the society recently helping to establish PAGs relating to XLH and chromosomal abnormalities.

Recommended call to action

Ensure that disease-specific rare disease support exists through the establishment of specialized PAGs supported by consolidated PAGs

Rationale: Consolidated PAGs are necessary to enhance the patient voice, while disease-specific PAGs are needed to identify disease-specific challenges and provide individualized support. Consolidated PAGs have the visibility, knowledge, experience, and financial capabilities to help establish and support smaller, disease-specific PAGs and should therefore take an active role in doing so.

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