2023

Pfizer

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MULTISTAKEHOLDER SUMMIT ON ADULT-ONSET RARE DISEASES

OUTPUT SUMMARY

Summit II – Lisbon 19th-20th October

Date of preparation: January 2024 - PP-UNP-GLB-1835

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INTRODUCTION

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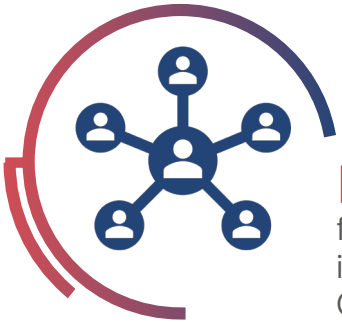
SUMMIT OBJECTIVES



BUILD an action plan to **DRIVE policy change** for adults living with rare diseases at a country level, using inspiration from the resources developed by the COLLABORATE Community

SHARE best practice examples of successfully advocating for policy change globally

IDENTIFY and PRIORITIZE challenges at specific points **across the life course** for people living with rare diseases

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SUMMIT AGENDA

DAY 1 - MORNING

Topic	Speaker
Welcome & introduction	*G. Mayman P. Teixeira*
COLLABORATE Summit I recap • Identified challenges and key learnings from Summit I • Examples of local multistakeholder collaboration in rare diseases	*A. Zygmunt* *N. Hanim Abdul Latif* *A. Tan*
Keynote presentations • Policy perspective • Patients' rights to access care • Decade of healthy ageing	*R.B. Leite* *M. Votta* *L. Tamblyn-Watts*
Coffee break	
COLLABORATE Summit I • Introduction to the identified resources	*S. Upadhyaya* *D. Wong-Rieger* *S. Berglund* *N. Hanim Abdul Latif* *A. Anastasakis*
Case study presentation: • Successful patient advocacy for driving policy change	*S. Shurtz*

DAY 1 - AFTERNOON

Topic	Speaker
Rotating working sessions • Building your action plan to drive policy change in your country	*Working group representatives*
Coffee break	
Interactive plenary • Driving consensus on unmet needs in adult-onset rare diseases	*S. Upadhyaya* *L. Tamblyn-Watts* *J. Gierczynski* *E. Berrios Barcenas* *D. Wong-Rieger*
Day 1 closure	*G. Mayman*

DAY 2

Topic	Speaker
Welcome & Introduction	*Moderator*
Keynote presentations: • Potential for AI • Ageing with a rare disease	*P. Magni* *J. De Graaf*
Working session • Identifying challenges across the life course for people living with rare diseases	*Working groups*
Coffee break	
Back presentation • Identifying challenges across the life course for people living with rare diseases	*Working group representatives*
Panel discussion • How can we ensure policy frameworks meet the specific needs of rare disease patients across the life course?	*L. Tamblyn-Watts* *P. Magni* *D. Wong-Rieger* *J. Young Maloney* *J. De Graaf*
Closure and next steps	*G. Mayman*

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In **October 2023**, Pfizer sponsored a meeting with **56 attendees** including patients, patient advocacy groups (PAGs), caregivers, healthcare professionals (HCPs) and policy experts who came together in Lisbon for the **second COLLABORATE Rare Disease Multistakeholder Summit**

They discussed how to drive change in adult-onset rare disease, **building local action plans** to change policy while **identifying and prioritizing challenges** across the life course for people living with rare diseases

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KEY EXPECTATIONS
FROM ATTENDEES AT THE MEETING

COLLABORATION
Crucial to share experiences and cooperate to make an impact in rare disease

FOCUS
on ageing

LEAVING AS FRIENDS
Creating a community that can be relied on for support, resource sharing and networking

Create
ACTION PLANS
for local implementation

SHARING OF IDEAS,
challenges and solutions

LEARN
from experiences in different countries, regions and disease areas

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THE «COLLABORATE» PANEL

Stefanie Bockwinkel
Master of Ceremony

Nadiah Hanim Abdul Latif
President, Malaysian Rare Disorders Society - Regional Representative, Southeast Asia, Oman, Qatar, South Korea - Phelan McDermid Syndrome Foundation

Durhane Wong-Rieger
President & CEO at Canadian Organization for Rare Disorders (CORD)

Jakub Gierczyński
Institute of Health Management

Alex Tan
Cardiologist - Clinical Interest: Cardiomyopathy, Heart Failure, Echocardiography - National Heart Centre Singapore

Mariano Votta
Director of Active Citizenship Network - Cittadinanzattiva

Sheela Upadhyaya
Life Sciences Consultant, specializing in Rare Diseases

Susanne Berglund
Clerk, FAMY Norrbotten association, Sweden

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THE «COLLABORATE» PANEL

Aris Anastasakis
Consultant Cardiologist and Specialist in inherited and rare cardiovascular diseases

Laura Tamblyn Watts
CEO at CanAge - Canada's National Seniors' Advocacy Organization

Paolo Magni
Professor at Università degli Studi di Milano and Italian Heart Foundation

Enrique Alexander Berrios Barcenas
Internal medicine – Cardiology. Instituto Nacional de Cardiología Ignacio Chávez

Jennifer Young Maloney
Vice President, Global Policy and Public Affairs – Specialty Care Pfizer

Johan de Graaf
Chair of the Dutch Pituitary Foundation and Endo-ERN ePAG

Ricardo Baptista Leite
CEO of HEALTH.AI and President of UNITE Parliamentarians Network for Global Health

DRIVING CHANGE IN ADULT-ONSET RARE DISEASES

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This section includes output from **two sessions** from the COLLABORATE Summit II

BUILDING A COUNTRY ACTION PLAN
TO DRIVE POLICY CHANGE

DRIVING CONSENSUS ON UNMET NEED
IN ADULT-ONSET RARE DISEASES

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The first working session used the resources developed by the working groups after Summit I as inspiration for participants to build their own action plan

Challenge identified in Summit I	A lack of advocacy capacity and funding for rare disease organizations (especially for older adults)	A lack of person-centered care for people living with adult-onset rare diseases	A lack of cohesion within the multidisciplinary ecosystem leading up to rare disease diagnosis (especially for adult-onset rare diseases)
Resource developed by working groups	Best practice toolkit for advocating for adult-onset rare diseases	Holistic care framework for adult-onset rare diseases	Tools and guidance to encourage stakeholder collaboration for adult-onset rare diseases
Summit II working session	Build your action plan to drive policy change using the resources as inspiration		

Participants were asked to consider the **policy change** that they want to drive **for adults living with rare diseases in their country**

They used this workbook to take note of how these resources may support them to build **their own action plan** to drive policy change in their country

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BUILDING YOUR ACTION PLAN TO DRIVE POLICY CHANGE IN YOUR COUNTRY USING THE BEST PRACTICE TOOLKIT FOR ADVOCATING FOR ADULT-ONSET RARE DISEASES

CHALLENGE AREA

Overcome barriers to rare disease advocacy, particularly for older adults, by equipping rare disease organizations with the knowledge and tools to successfully advocate for people with adult-onset rare diseases

RESOURCE DISCUSSED IN THE SESSION

This toolkit was created to support patient organizations in their engagement with key stakeholders across the rare disease community on the topic of adult-onset rare diseases. For each stakeholder type, it includes key discussion points for engagement, a call-to-action, and opportunities for engagement

AT THE END OF THE SESSION ATTENDEES WERE ASKED

“HOW HAS THIS SESSION INSPIRED YOU TO DRIVE POLICY CHANGE FOR ADULTS LIVING WITH RARE DISEASES IN YOUR COUNTRY?”

KEY THEMES FROM THE DISCUSSION INCLUDED

- There is a need for better data generation & collection for adult-onset rare diseases, including the impact of rare diseases on ageing
- Rare diseases should be integrated into social policies and services to ensure patients have access to everything that is needed to manage their condition
- Adult-onset rare diseases need to be on the national rare disease plan/strategy
- There remains a need for better awareness of and education on adult-onset rare diseases across all stakeholders, including the need for caregiver support
- HCP education, including rare disease programs at medical schools and at a post-graduate level, are required; there is a need for more rare disease specialists and centers of excellence
- Rare disease patient organization representatives from smaller countries should create an alliance to develop common guidance for implementation

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BUILDING YOUR ACTION PLAN TO DRIVE POLICY CHANGE IN YOUR COUNTRY USING THE HOLISTIC CARE FRAMEWORK FOR ADULT-ONSET RARE DISEASES

CHALLENGE AREA

Ensure that patients, particularly those whose disease onsets in adulthood, are treated holistically as people, by informing them of all possible options for care and support and involving them in decision-making

RESOURCE DISCUSSED IN THE SESSION

This holistic care framework was created to standardize the approach to holistic care provision for adults with rare diseases. It includes key topics for HCPs to consider in discussions with adult-onset rare disease patients to ensure holistic, person-centered care is delivered

AT THE END OF THE SESSION ATTENDEES WERE ASKED

“HOW HAS THIS SESSION INSPIRED YOU TO DRIVE POLICY CHANGE FOR ADULTS LIVING WITH RARE DISEASES IN YOUR COUNTRY?”

KEY THEMES FROM THE DISCUSSION INCLUDED

- **Education for doctors and public –** create reference centers/guidelines, consider patient well-being, support workers, create a patient journey
- **Reduce time to diagnosis** – gather and better utilize rare disease-specific data to help speed up diagnosis
- **Better connect expert multidisciplinary team (MDT)** – better connect patient systems, collaboration between health, social and educational institutions
- **Reduce barriers to access of treatments and data** – allow access to treatments across borders, share access to patient data across the MDT
- **Better patient support** – support following diagnosis, reduce separate clinic visits, support groups, helpline, patient case managers
- **Policy change** – include adult-onset rare diseases in national rare disease plans, create a national rare disease office and charter of patient rights, presence of all stakeholders in decision-making, better use of data

GP: general practitioner; **HCP:** healthcare professional; **MDT:** multidisciplinary team.

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BUILDING YOUR ACTION PLAN TO DRIVE POLICY CHANGE IN YOUR COUNTRY USING THE TOOLS AND GUIDANCE TO ENCOURAGE STAKEHOLDER COLLABORATION FOR ADULT-ONSET RARE DISEASES

CHALLENGE AREA

Speed up and improve diagnostic accuracy for people with adult-onset rare diseases through improved multistakeholder collaboration

RESOURCE DISCUSSED IN THE SESSION

This resource was developed to provide the rare disease community with tools and strategic guidance to encourage collaboration across the ecosystem. It consists of two parts:

- A strategic approach for involving stakeholders at all levels starting at national (regional) level to build up to EU/international level
- 'Meeting-in-a-box' guidance for the meetings' organization and coordination to ensure the participation of the right stakeholders at every step
- The resource was developed with a focus on amyloidosis but is applicable to other rare diseases

AT THE END OF THE SESSION ATTENDEES WERE ASKED

"HOW HAS THIS SESSION INSPIRED YOU TO DRIVE POLICY CHANGE FOR ADULTS LIVING WITH RARE DISEASES IN YOUR COUNTRY?"

KEY THEMES FROM THE DISCUSSION INCLUDED

- **From countries outside the EU or without ERN's support**
  - **Strengthen the role of patient organizations**
  - **Ministry of Health involvement** – to sign a "Lisbon letter for the rights of rare disease patients" for a stakeholder roundtable at the Ministry of Health (MoH)
  - **Decentralization of services and treatment** for a better cooperation between PAGs, doctors, distributors of medicines, MoH, Media
  - **Increase funding** for medical treatment
- **From EU countries with ERN's support**
  - **Yearly multistakeholder event** in the country bringing together actions from policymakers, decision-makers, HCPs, PAGs, pharmaceutical industry
  - **Raising awareness** about the role and work of **Centers of Expertise (reference centers) and cooperation with ERN**.
  - **EU financing of ERN and reference centers** in every EU country; monitor outcomes
  - Set up **national (and/or local) group + European group** to transmit needs to the EU (how to create a European group)
  - **ERN standardized approach** to include clear, defined objectives to communicate with patient organizations (aligned on methodology and guidelines)

ERN: European Reference Network; **EU:** European Union; **HCP:** healthcare professional; **PAG:** patient advocacy group.

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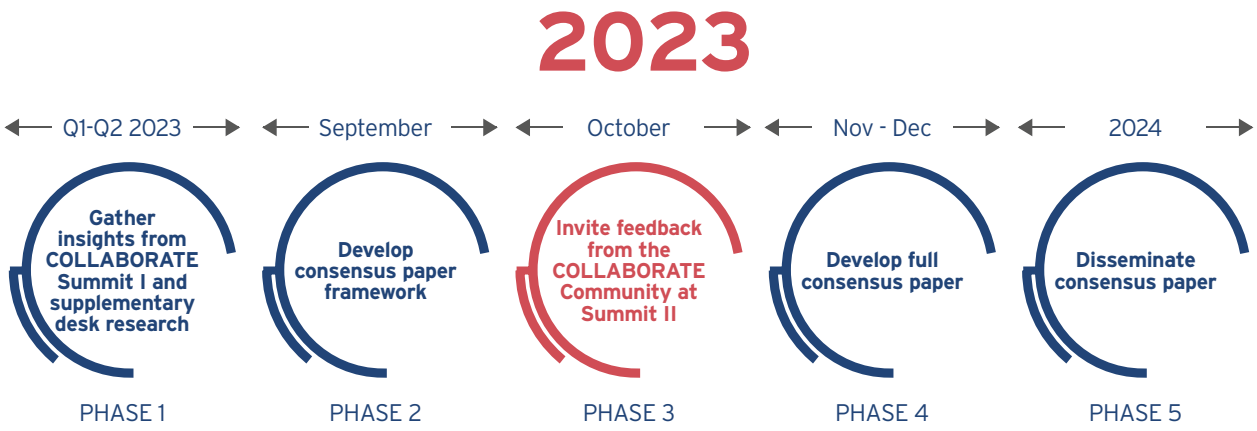
PARTICIPANTS WERE INTRODUCED TO THE **CONSENSUS PAPER** THAT IS CURRENTLY UNDER DEVELOPMENT

Since the first COLLABORATE Summit, a small multistakeholder steering committee has been working with Pfizer to develop a consensus paper outlining challenges and opportunities for adult-onset rare diseases



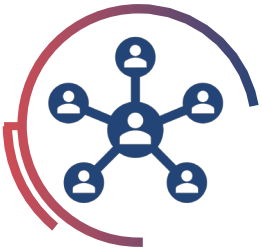
PARTICIPANTS WERE GIVEN THE OPPORTUNITY TO PROVIDE **FEEDBACK** ON THE PROPOSED CHALLENGE AREAS IN THE CONSENSUS PAPER

The consensus paper titled 'Challenges & opportunities in adult-onset rare diseases: perspectives from a global multistakeholder expert group' aims to:

- Consolidate the insights from the first COLLABORATE Summit and the subsequent 2023 working group meetings
- Outline the key challenges, potential solutions, and a clear policy-focused call-to-action for adult-onset rare diseases

During this session we...

...introduced
the consensus paper to the wider COLLABORATE group



...discussed
the need for consensus in adult-onset rare diseases

...obtained feedback
on the key challenge areas identified for adult-onset rare diseases

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PARTICIPANTS **VOTED** ON THE SIGNIFICANCE OF THE CHALLENGE STATEMENTS WITHIN OUR **THREE CHALLENGE AREAS**

The top **2 most significant challenges** from each challenge area were:

Delays to diagnosis of adult-onset rare diseases

1. Many primary care physicians (PCPs) lack awareness and understanding of rare diseases and may not recognize symptoms following presentation of a patient to the healthcare system
2. A lack of public awareness of rare diseases generally, and especially those with an adult onset, can lead to delayed recognition of symptoms and subsequent delay to presentation to the healthcare system

"You keep going back to the doctor and get the same story each time, you are not listened to, are dismissed and you give up"

Unequal access to adequate treatment for adult-onset rare diseases

1. Expertise in rare diseases is limited, with HCPs lacking specific training and education on rare diseases and how to treat them, which can impact the level of care provided to patients
2. The treatment of rare diseases is complex and challenging, especially for those with adult-onset disease, where age-related comorbidities must also be taken into account

"Rare diseases is not only a topic for pediatricians, but now for many other doctors and this is new across the world"

Inadequate coordinated care and support for people living with an adult-onset rare disease

1. There is a lack of policies and services to address the social, economic & psychological burden on families and caregivers of people with rare diseases, particularly for adult-onset rare diseases where specific challenges require further support
2. People with adult-onset rare diseases and their caregivers lack support across the entire patient pathway, from initial symptoms to long-term care

"It is important where a person is given their care. Is it a center of expertise with an MDT team, or is it a peripheral hospital where there is no knowledge of rare diseases?"

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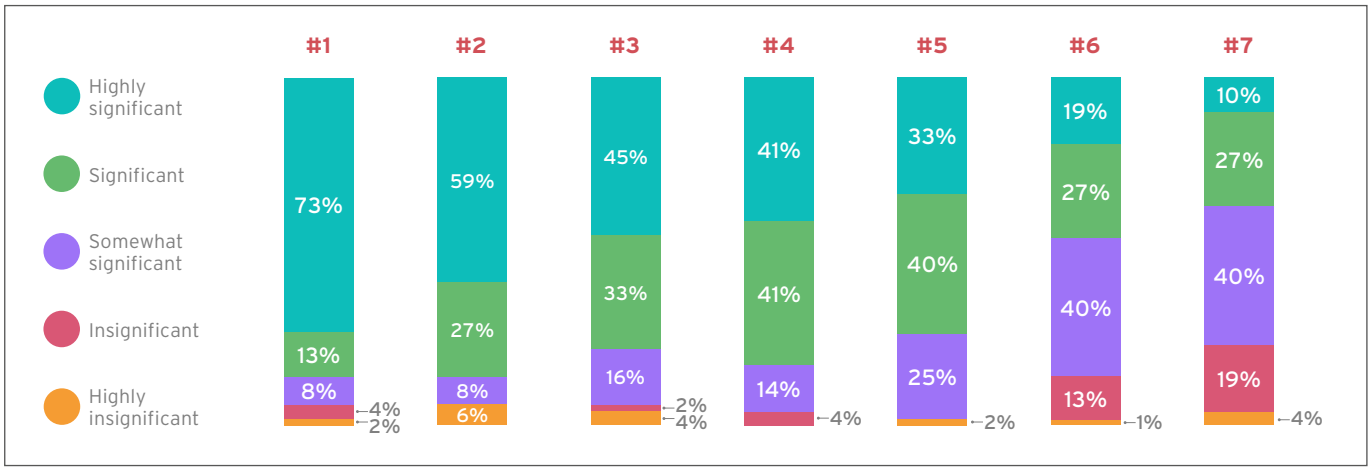
AREA 1

DELAYS TO DIAGNOSIS OF ADULT-ONSET RARE DISEASES

How significant is this challenge for people living with adult-onset rare diseases in your country?



#1: Many primary care physicians (PCPs) lack awareness and understanding of rare diseases and may not recognize symptoms following presentation of a patient to the healthcare system.

#2: A lack of public awareness of rare diseases generally, and especially those with an adult onset, can lead to delayed recognition of symptoms and subsequent delay to presentation to the healthcare system.

#3: Even if symptoms are suspected by the patient, a low sense of self-worth regarding their health may mean that older adults delay visiting a healthcare professional.

#4: Patients with adult-onset rare diseases are more likely to have their symptoms dismissed by HCPs as being a part of ageing, resulting in delayed diagnosis.

#5: There is a lack of cohesion/linked infrastructure for the diagnosis of rare diseases at the healthcare system level, with inconsistencies between provision for childhood rare diseases versus adult-onset rare diseases.

#6: Adults may delay reporting their symptoms to an HCP due to the potential impact of a rare disease diagnosis on social and romantic relationships, finances, and their ability to gain or maintain employment, or obtain coverage.

#7: In certain countries and cultures rare diseases are associated with stigma and taboo, which can undermine the willingness to seek screening, with some adults preferring not to know if they have a rare disease.

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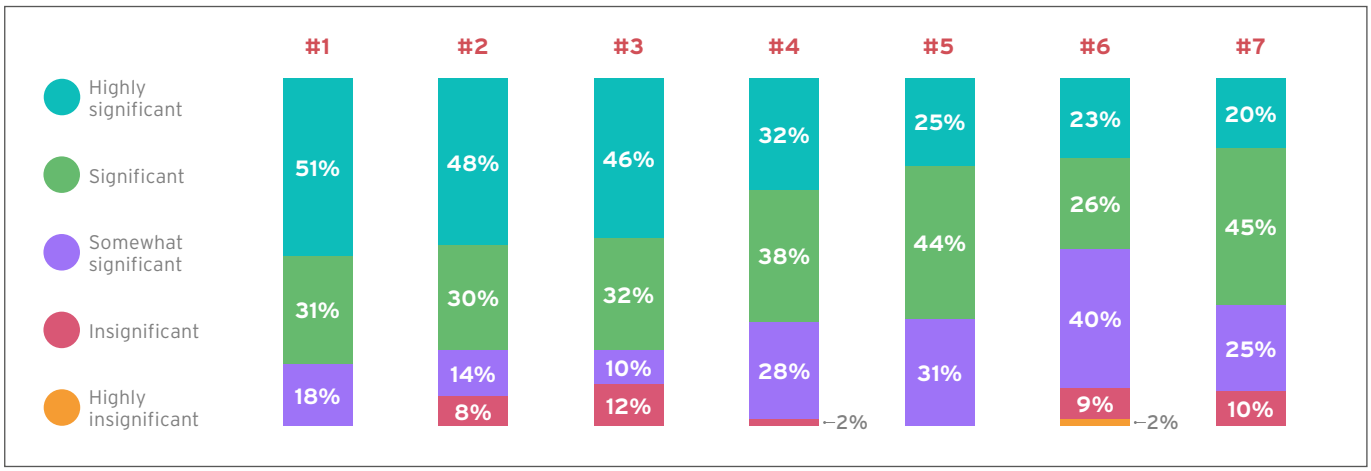
AREA 2

UNEQUAL ACCESS TO ADEQUATE TREATMENT FOR ADULT-ONSET RARE DISEASES

How significant is this challenge for people living with adult-onset rare diseases in your country?



#1: Expertise in rare diseases is limited, with HCPs lacking specific training and education on rare diseases and how to treat them, which can impact the level of care provided to patients.

#2: The treatment of rare diseases is complex and challenging, especially for those with adult-onset disease, where age-related comorbidities must also be taken into account.

#3: Many patients lack awareness of government decision-making processes on access (e.g., HTA, reimbursement, and cost-sharing), which can be a barrier to effective patient advocacy, particularly for older adults who are less likely to self-advocate.

#4: Funding, research and advocacy efforts tend to be weighted towards childhood rare diseases, leaving development of treatments for adult-onset rare diseases lagging behind.

#5: Access to rare disease treatment is limited, with treatments less likely to receive marketing authorization compared with drugs for non-orphan diseases and the combination of high costs with weak and often lacking data limiting reimbursement.

#6: Many countries do not have rare disease policies and, where policy frameworks do exist, they are often weighted towards childhood rare diseases, which hinders prioritization of initiatives to improve access to care for adult-onset rare diseases.

#7: The low prevalence and subsequent lack of research of rare diseases has hindered development of treatment guidelines, and although national and local guidelines are being developed in a small number of countries, they are not widely implemented.

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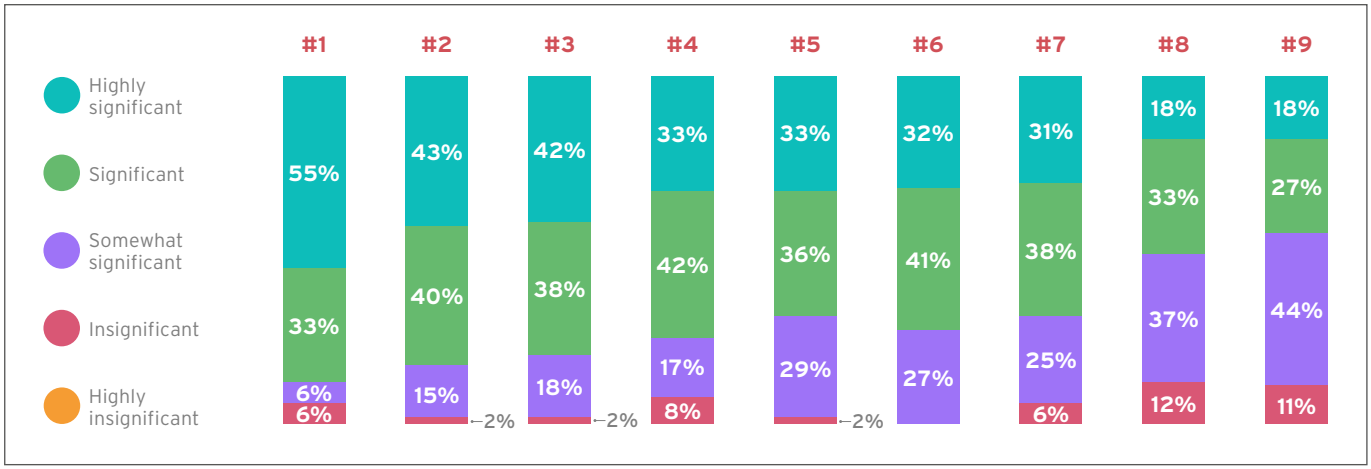
AREA 3

INADEQUATE COORDINATED CARE AND SUPPORT FOR PEOPLE LIVING WITH AN ADULT-ONSET RARE DISEASE

How significant is this challenge for people living with adult-onset rare diseases in your country?



#1: There is a lack of policies and services to address the social, economic & psychological burden on families and caregivers of people with rare diseases, particularly for adult-onset rare diseases where specific challenges require further support.

#2: There is a particular need for coordinated multidisciplinary care for adult-onset rare diseases, including specialists in geriatrics and comorbidities commonly associated with ageing.

#3: People with adult-onset rare diseases and their caregivers lack support across the entire patient pathway, from initial symptoms to long-term care.

#4: Clear and simple education resources are lacking for people with adult-onset rare diseases and their caregivers, with those that do exist often tailored for parents of children with rare diseases.

#5: Coordinated, multidisciplinary care is lacking for people with rare diseases generally, but where this does exist it is weighted towards diseases diagnosed in childhood.

#6: The rare disease landscape is complex and navigation information for patients is lacking, which can impede their confidence and ability to self-advocate and manage their own condition.

#7: Older patients regularly experience ageism and paternalism while navigating healthcare and attempting to advocate for themselves.

#8: While patient associations, educational websites, and peer-to-peer support all exist for rare disease patients, they are often inadequate, and many are not relevant to those with adult-onset rare diseases.

#9: HCPs may be less likely to deliver person-centered care for people with adult-onset rare diseases or engage in shared decision-making.

IDENTIFYING CHALLENGES

ACROSS THE LIFE COURSE FOR PEOPLE LIVING WITH RARE DISEASES

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For the second part of the Summit, we pivoted to focus on rare diseases across the life course and this section includes the output from the following two sessions

WORKING SESSION

«Identifying challenges **across the life course** for people living with rare diseases»

INTERACTIVE PANEL DISCUSSION

«How we can ensure policy frameworks meet the specific needs of rare disease patients **across the life course**»

PARTICIPANTS IDENTIFIED AND PRIORITIZED KEY CHALLENGES FACED BY PEOPLE LIVING WITH RARE DISEASES ACROSS THE LIFE COURSE

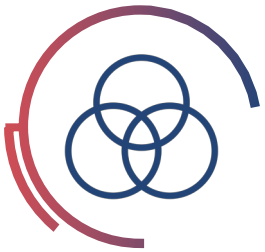
OBJECTIVE

Identify and prioritize challenges at specific points across the life course for people living with rare diseases

WORKING SESSION

What are the challenges for people living with rare diseases across the life course?

Which are the top 2 challenges for people living with rare diseases in your country?



How do your priorities differ from your peers from other countries? Can you align?

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PRIORITIZED CHALLENGES - GROUP 1

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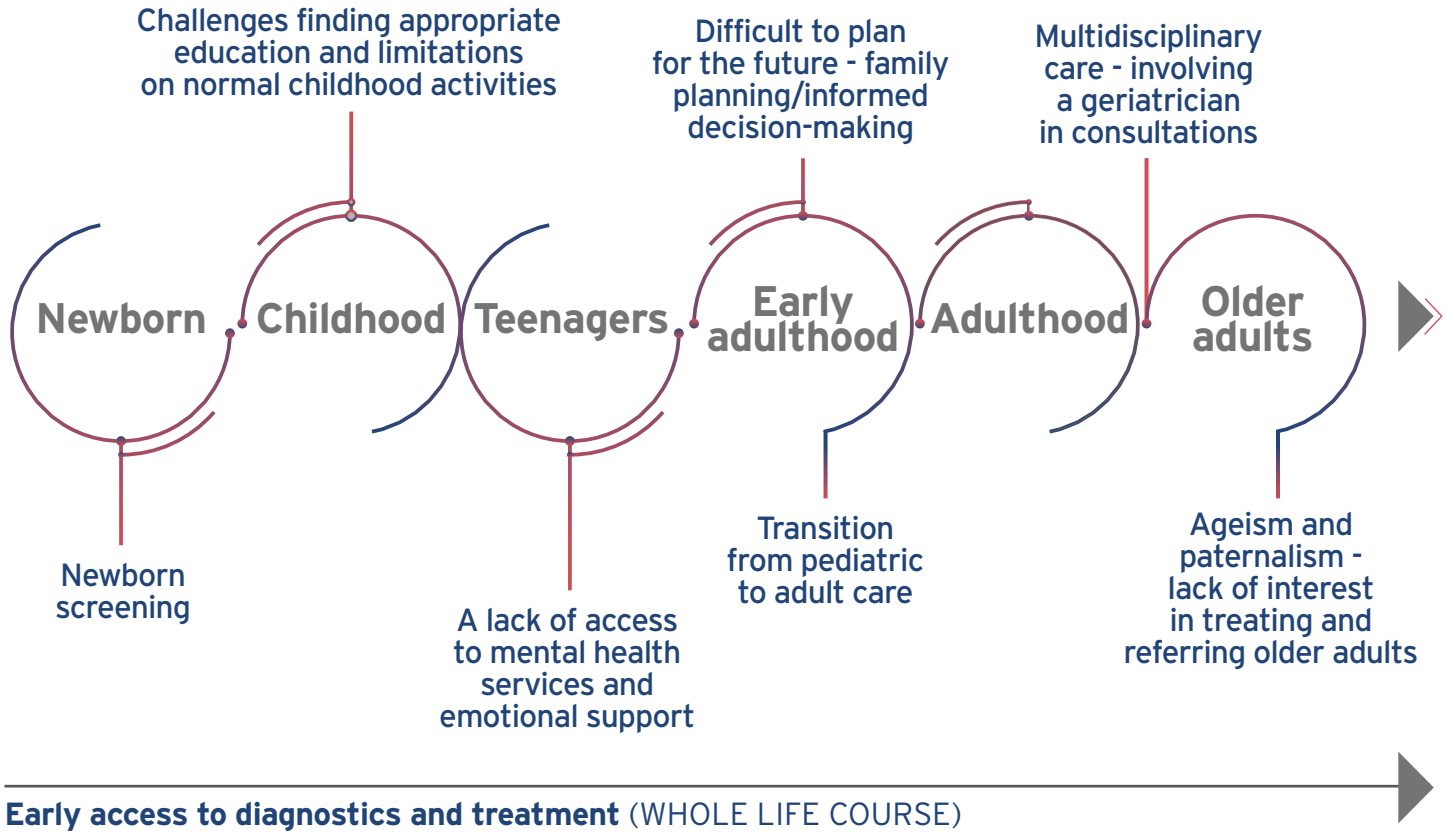
COUNTRIES: Bosnia and Herzegovina, Canada, France, North Macedonia, Poland, Portugal, Romania, UK

KEY CHALLENGES AT SPECIFIC TIME POINTS ACROSS THE LIFE COURSE FOR PEOPLE LIVING WITH RARE DISEASES



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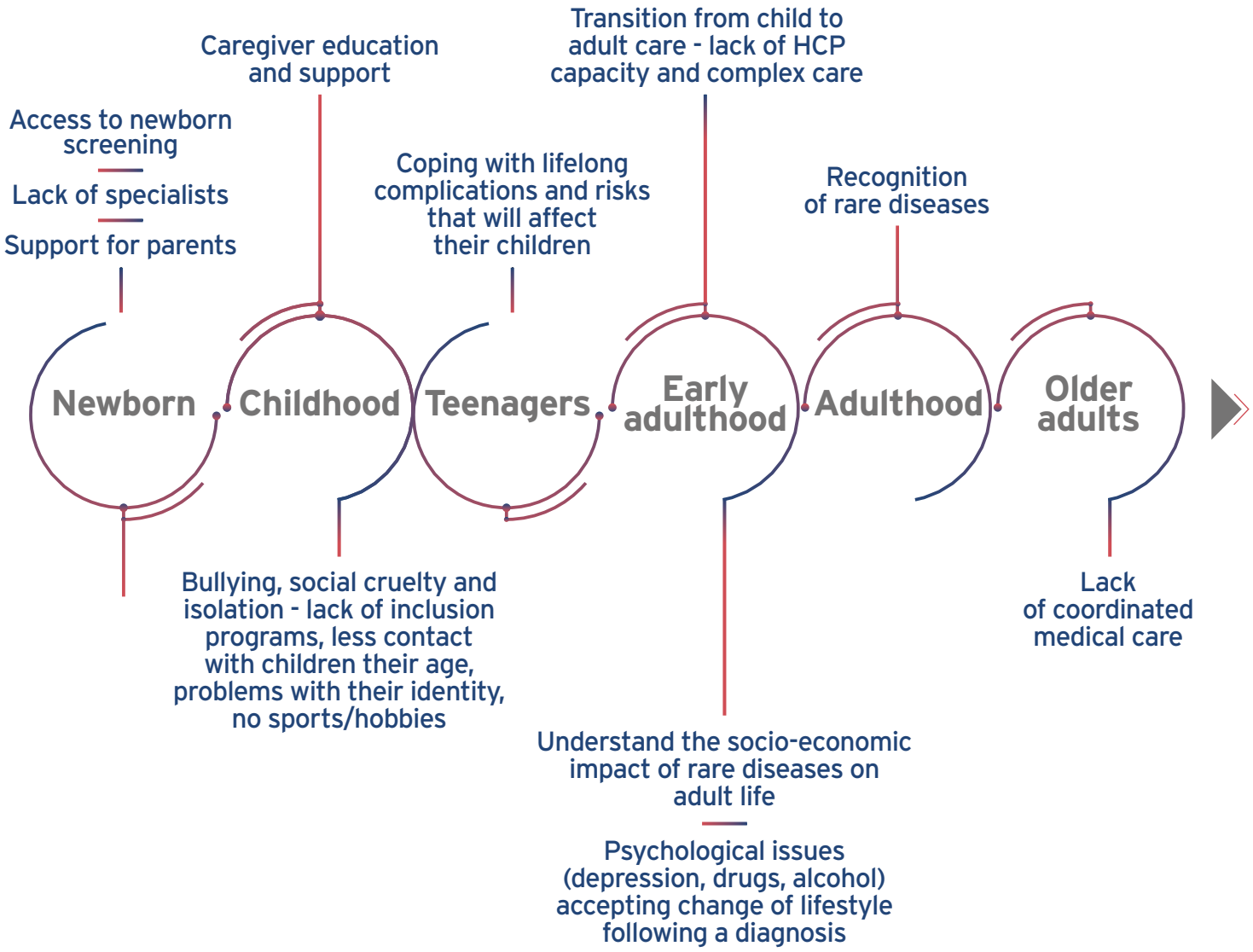
PRIORITIZED CHALLENGES - GROUP 2

COLLABORATE SUMMIT 2023

COUNTRIES: Argentina, Belgium, Brazil, Colombia, Croatia, Czech Republic, Ireland, Mexico, Netherlands, Slovenia, Sweden

KEY CHALLENGES AT SPECIFIC TIME POINTS ACROSS THE LIFE COURSE FOR PEOPLE LIVING WITH RARE DISEASES



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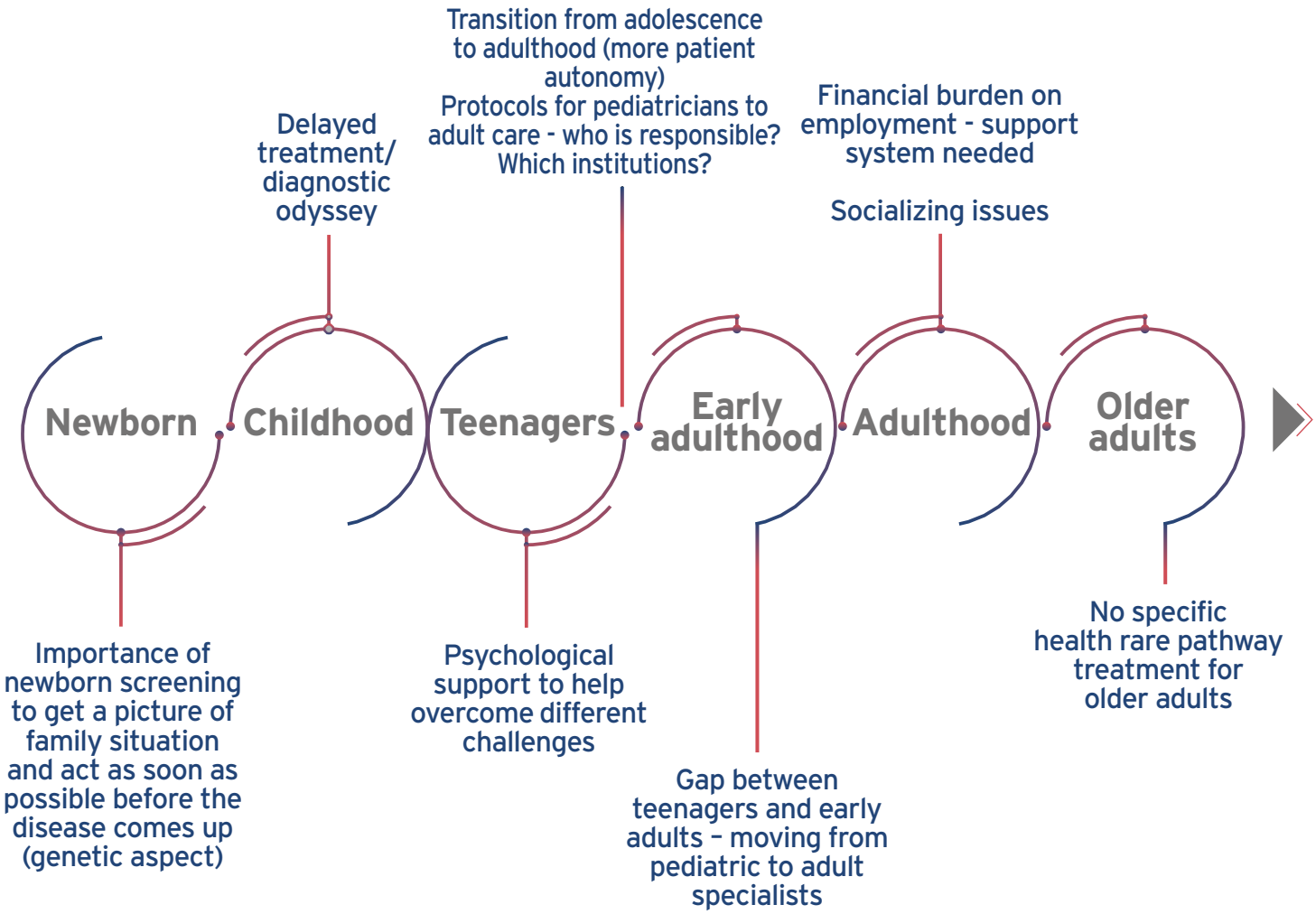
PRIORITIZED CHALLENGES - GROUP 3

COLLABORATE SUMMIT 2023

COUNTRIES: Bahrain, Cyprus, Denmark, Finland, Greece, Italy, Kazakhstan, Malaysia, Serbia, Singapore, Spain, Ukraine

KEY CHALLENGES AT SPECIFIC TIME POINTS ACROSS THE LIFE COURSE FOR PEOPLE LIVING WITH RARE DISEASES



Disease awareness/rare disease awareness overall across life course
Fight misinformation about the disease (WHOLE LIFE COURSE)

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SUMMIT II CLOSED WITH A PANEL DISCUSSION FOCUSED ON POLICY FRAMEWORKS FOR RARE DISEASE PATIENTS ACROSS THE LIFE COURSE

KEY TAKEAWAYS

ENSURE POLICY FRAMEWORKS MEET THE SPECIFIC NEEDS OF RARE DISEASE PATIENTS ACROSS THE ENTIRE LIFE COURSE

- When patients are screened, ensure both medical treatment and a reset of where you are in the life course are triggered
- Doctors say that a patient's QoL is impacted only by their hormone levels, but there is much more to think about, and it is our job to make doctors aware of these other factors that influence patient QoL
- Urgency – for rare disease patients, time is life. We need to help policymakers understand that we need to act now
- Demonstrate why allocations towards rare disease should be seen as an investment, rather than an expense
- Don't neglect older people, treat them with the same care as you would a child

HOW TO ADDRESS THE LIFE COURSE

- Add language around longevity to policy conversations. Ensure rare diseases are included in planning for the demographic shift
- Stop thinking in silos. Someone lost because of a rare disease is also a loss for society
- We don't treat diseases or conditions, we treat people. We need to personalize care

ENSURE THAT WE HEAR VOICES FROM PEOPLE ACROSS THE LIFE COURSE

- Rare disease stigma is high, making it difficult for patients to speak up
- If one person speaks up, it then empowers others. We need to celebrate those heroes
- Patients are critical to understanding the disease and articulating it for others
- Process of creating policy change is too long compared to the term of a politician

EXISTING POLICY FRAMEWORKS AND OTHER DISEASE AREAS WE CAN LEARN FROM

- Cancer affects all ages. The narrative focuses on impacts at certain life events
- Dementia can also be used to speak about the life course. When screening for dementia, there is an opportunity to also screen for other diseases
- Drawing more connectivity with other disease area communities can help understand how they approach the life course
- With HIV, how did they progress from a stigmatized disease, to a chronic disease?

OTHER THOUGHTS

- Technology usage in older people has increased but there is an access problem. We are improving attitudes and comfort of technology use in older people
- Create a commission on bioethics focused on the patient perspective
- Be mindful of the other countries not represented here and prioritize solutions that work for your country
- Deep dive on training and capacity building for advocacy leaders

QoL: quality of life.

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CLOSING REMARKS

In an ideal world, with an ideal policy framework, that utilizes the life course concept. What do people with rare diseases experience in this world?

- Normalization – no stigma, something HCPs are used to, MDT team well established and normalization of psychosocial and economic impact
- Acceptance
- Policy frameworks that support QoL. The right enabling environment to support patient access and QoL
- Patients with a disease, rare or not, with the best diagnosis, care and social acceptance which should be the same for every disease

HCP: healthcare professional; **MDT:** multidisciplinary team; **QoL:** quality of life.

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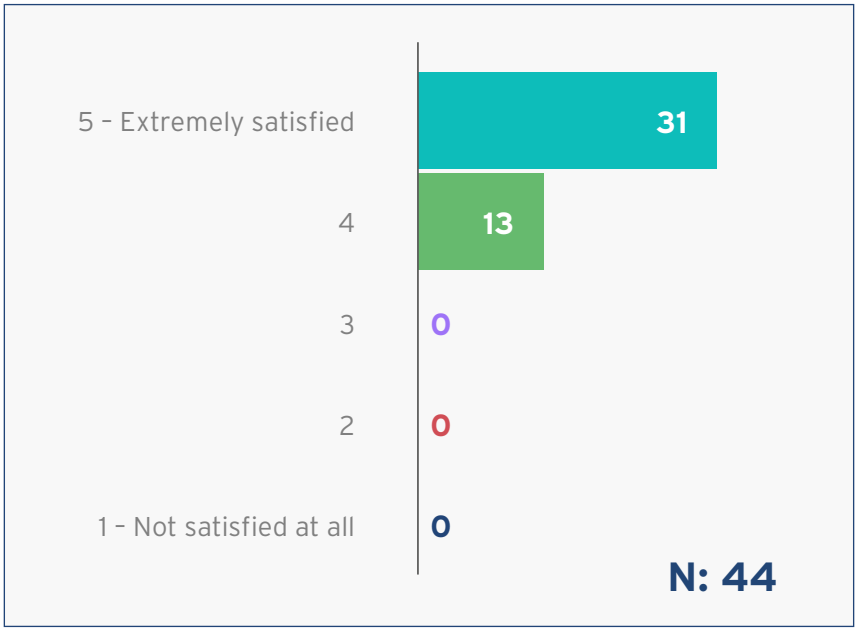
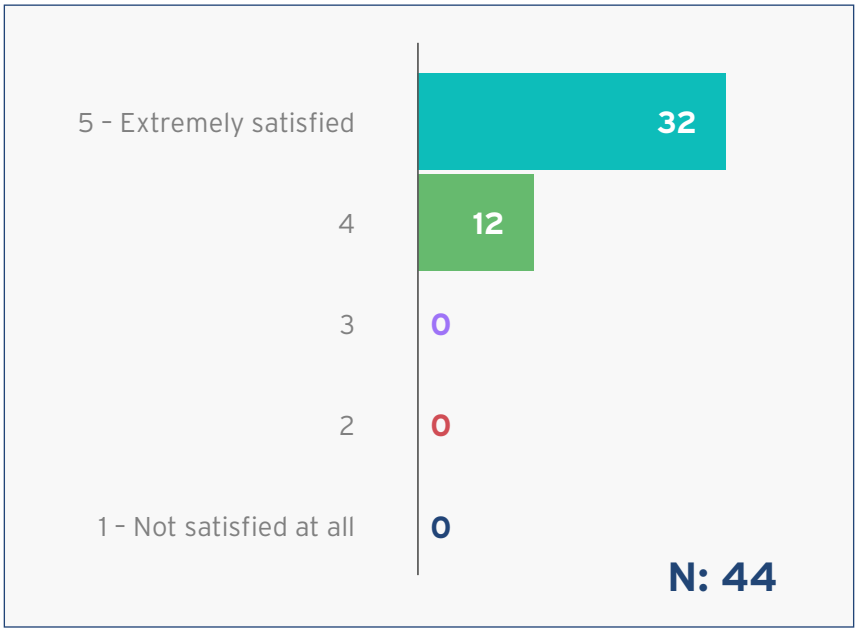
RESULTS FROM THE «SUMMIT FEEDBACK FORM» REPORT
CONSOLIDATED FEEDBACK FROM 44 COMPLETED EVALUATION FORMS

HOW SATISFIED ARE YOU WITH THE SUMMIT FORMAT?
(multidisciplinary meeting with panel and workshop sessions)



HOW SATISFIED ARE YOU WITH THE TOPICS COVERED DURING THE SUMMIT?



CONCLUSION AND NEXT STEPS FOR COLLABORATE

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Working together for adult rare diseases

To continue to grow the momentum of the COLLABORATE Summit II, we call on all stakeholders to share knowledge, raise awareness, and ensure the representation of older adults in the rare disease space

WE CALL ON ALL STAKEHOLDERS TO COLLABORATE TO

- **Advocate for enactment/implementation of the specific policy change** that you determined you want to drive in your country for adults living with rare diseases
- **Be prepared in 2024** to come together to ideate solutions to our prioritized challenges across the life course for people living with rare diseases
- **Action the opportunities** for change that have been identified by the groups

AS A FOUNDATION FOR ADDRESSING THE BELOW CALLS-TO-ACTION, PFIZER IS COMMITTED TO

- **Supporting** the ongoing COLLABORATION between stakeholders
- **Working with stakeholders** to develop an actionable roadmap for 2024 and beyond
- **Finalizing the consensus paper** that consolidates the insights from the first COLLABORATE Summit and the subsequent 2023 working group meetings and outlines the key challenges, potential solutions, and a clear policy-focused call-to-action for adult-onset rare diseases

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KEY OPPORTUNITIES FOR CHANGE

IMPROVE AWARENESS OF AND EDUCATION ON ADULT-ONSET RARE DISEASES ACROSS ALL STAKEHOLDERS

- Educate HCPs at medical school and post-graduate level on rare diseases to increase the number of specialists
- Create reference centers and guidelines for GPs to help HCPs remain up to date on the latest advances
- Organize multistakeholder events to bring together all stakeholders to solve outstanding issues – at a country/regional level policymakers, decision-makers, HCPs, PAGs, pharma

INTEGRATE ADULT-ONSET RARE DISEASES INTO THE NATIONAL RARE DISEASE PLAN/STRATEGY AND SOCIAL POLICIES/SERVICES

- Create a charter of patient rights with patients and the ministry of health to ensure that adult-onset rare diseases are included in disease plans and all stakeholders have a say in decision-making

INCREASE DATA GENERATION ON ADULT-ONSET RARE DISEASES AND THE IMPACT OF RARE DISEASES ON AGEING

- Drive an increase in funding for treatments and resources for patient support
- Better understand signs and symptoms of adult-onset rare diseases and the impact of rare diseases on older patients to improve diagnosis times

STRENGTHEN THE ROLE OF RARE DISEASE PATIENT ORGANIZATIONS

- Create an alliance of PAGs from smaller countries to develop common guidance for patients
- Better support patients following diagnosis by reducing clinic visits, creating support groups, helplines and patient case managers/support workers

DECENTRALIZE TREATMENT AND SERVICES TO ALLOW FOR BETTER COOPERATION BETWEEN ALL STAKEHOLDERS

- Better connect the expert multidisciplinary team through patient systems and collaboration between health, social and educational institutions
- Create a standardized ERN approach with clear objectives to communicate with patient organizations

ERN: European Reference Network; **GP:** general practitioner; **HCP:** healthcare professional; **PAG:** patient advocacy group.


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