

Policy statement on rare diseases in the Nordic countries

Purpose of this Policy Statement

This policy statement has been developed by Takeda Pharma, Novo Nordisk, Sobi, and Alexion AstraZeneca Rare Disease, the four industry partners involved in the organization of the [Nordic Rare Disease Summit](#)¹ which took place in Stockholm on April 17, 2023. We share our commitment to a better rare disease landscape and look forward to a continued partnership with all the Summit co-organisers (Rare Diseases Sweden, lif (the research-based pharmaceutical industry), the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), the European Federation of Pharmaceutical Industries and Associations (EFPIA), EURORDIS Rare Diseases Europe, and SBONN (Sällsynta Brukarorganisationers Nordiska Nätverk).

Based on this year's summit conclusions, we are sharing our initial policy recommendations on how to strengthen Nordic collaboration in the area of rare diseases. In order to improve the quality of care and outcomes for the one million people² living with a rare disease in the Nordics, as well as long-term benefits for individuals, caregivers and societies, we aim to foster a consistent exchange of best practices and experiences. The suggestions, which all deserve to be further unpacked, aim to spark a discussion at the upcoming [Nordic Conference](#) on Rare Diseases on 2-3 October 2023. We have, therefore, aligned the structure with the same four core topics put forward in the Conference agenda.

We have been inspired by recent efforts in the Nordic region, including the establishment in 2016 of the Nordic Network on Rare Diseases (NNRD) on behalf of the Nordic Council of Ministers, the Könberg report 'The Future Nordic Health Cooperation' as well as the past two Nordic Rare Disease Summits.

A notable recommendation from this year's Summit, which we welcomed, was for the development of a new Nordic Roadmap for Rare Diseases with measurable recommendations. This new Rare Disease Roadmap will take into account the discussions at the Nordic Conference in October, and will hopefully be agreed upon before the end of this year by all ten partner organisations of this year's Summit.

¹ The Nordic Rare Disease Summit brings together experts, decision-makers, policymakers as well as representatives from civil society, patient organizations, academia, and industry to elevate rare diseases as a priority area in the Nordic countries. So far, two Summits have been organized, in 2021 and 2023, and focused on early diagnosis, patient empowerment, access to innovation, and the EU Pharmaceutical Package. The first Summit resulted in the 2021 Nordic Roadmap for Rare Diseases. As a follow-up to the first roadmap, a new extended Nordic Rare Disease Roadmap will be available at the end of 2023. The next Rare Disease Summit is expected to take place in 2025, likely during the Danish Presidency of the Council of the EU.

² Center for Rare Diagnoses (CSD), Karolinska University Hospital



NATIONAL RARE DISEASE STRATEGIES:

We welcome all efforts to develop and implement rare disease strategies for the Nordic countries, and recognise the various stages of development across different Nordic countries – Denmark, Norway and Finland already have national plans in place; Sweden and Iceland recently announced work on their first national strategies. With that in mind, we invite stakeholders to consider how we can improve in the following areas:

- Systematic inclusion of patients in all processes in the development of plans
- Holistic, taking into account all aspects of living with a rare disease
- Platforms for collaboration between public and private stakeholders
- Multi-stakeholder governance (including patients and industry) to ensure regular monitoring, evaluation and revision of the strategies
- Explicit goals and allocated budget for the implementation of the strategies
- Inclusion of measures to improve early diagnosis, ensure patient involvement in policy shaping and securing access to innovation
- Incorporation of the work of European Reference Networks (ERNs) into national health care systems.

Further inspiration can be taken from the [Rare 2030 recommendations](#), the development of which was led by EURORDIS.

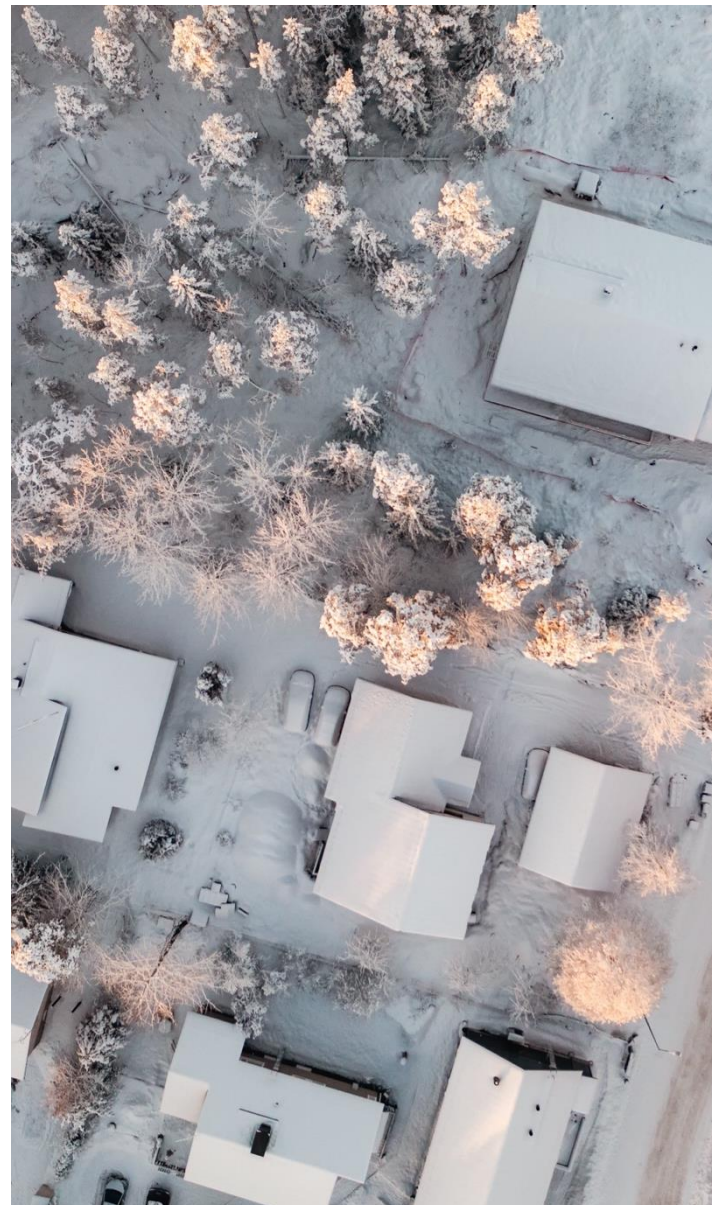


DIGITALISATION, DATA SHARING AND REGISTRIES:

The potential of health data and AI for rare diseases thus far has not been fully leveraged. Data on rare diseases is often scarce, fragmented, and inconsistent due to lack of registries or a consistent approach. The Nordic countries have historically been best-in-class in terms of patient registries and data collection. The Nordic countries could lead the way in Europe, showcasing best practice data collection and development of rare disease patient registries, which could enable clinical research, trials and improved diagnosis and treatment. With that in mind, we invite stakeholders to consider how we can improve in the following areas:

- Harmonised and common standards for data registries to harness the potential of cross-border data use. [RaraSwed kvalitetsregister](#) as a best practice

- Introduction of harmonised diagnosis coding. Implementation of [OrphaCodes](#) supported by the Finnish rare diseases programme as a best practice
- Interoperability to support data exchange between healthcare providers both within and across Nordic countries as well as secondary use of health data. The [European Health Data Space \(EHDS\)](#) presents an excellent opportunity to enable such exchange
- Optimal collection, generation and cross-border use of real-world evidence (RWE) in drug development, HTA and regulatory decision-making
- Updated and harmonised legislation regarding health data in line with security and privacy requirements for data while ensuring greater involvement of patients' perspective





COLLABORATIONS CROSS BORDERS:

No single country can, on its own, address the high unmet needs of patients with ultra-rare diseases, including the need for early diagnosis. We can do a lot more to improve collaboration in the Nordic region, by building on the impressive work already done by a variety of stakeholders, including patient organizations, public authorities, and companies. We invite stakeholders to consider how we can improve in the following areas:

- Strengthen the Nordic Network on Rare Diseases (NNRD), through increased dialogue and the support of Ministries of Health, as well as the Nordic Council of Ministers. The NNRD could become a platform for sharing progress and best practices on national rare disease plans
- Financial support and participation in cross-border initiatives such as the [SBONN](#)
- Foster collaboration between the European Reference Networks (ERNs) and industry and other researchers to further leverage the wealth of knowledge and data within the ERNs

We are confident that initiatives like the Nordic Conference on Rare Diseases will support the long-term vision of enabling better lives for the one million³ people living with rare diseases in the Nordic countries – and we look forward to discussing these ideas at the upcoming conference.

³ Center for Rare Diagnoses (CSD), Karolinska University Hospital

- Leverage the Nordic Pharmaceutical Forum to encourage the use of cross-border data-sharing, HTA assessment, and best practices. FINOSE, the HTA collaboration network between Fimea (Finland), NoMA (Norway) and TLV (Sweden) and DKMA (Denmark) is strong Nordic examples of cross-border HTA collaboration
- Improve the application and also raise patients' awareness of the EU cross-border healthcare directive, to ensure that people with rare diseases in the Nordic can fully profit from it



PRECISION MEDICINE:

Molecular diagnostics and precision medicine are particularly suited to diagnosing and treating rare diseases and overall can lead to better outcomes by giving the right treatment to the right person at the right time. Precision diagnosis can lead to quicker diagnosis, tailored treatments, reduced expenses, more effective clinical trials and development of novel medicines. With that in mind, we invite stakeholders to consider how we can improve in the following areas:

- Allocate additional budget to precision medicines, capturing a holistic view of long-term savings over the patient lifetime
- Dialogue between healthcare professionals, payers and manufacturers in order to develop outcome-based, and innovative financing, payment and reimbursement models to stimulate the development and uptake of precision medicine in the Nordics
- Bridge the divide between research and clinical practice
- Allocate financial resources towards research, development and technical expertise and infrastructure for precision medicine
- Increase the use of innovative diagnostics in hospitals across the Nordics, supported by adequate funding
- Encourage more collaboration between public and private partners, as well as patients and not-for-profits to find common ground between regulators, payers, companies, patients, and healthcare providers on how to handle orphan drugs and ATMPs, which often have limited evidence
- Engage patients, relatives and close ones in how we best shape this new paradigm



FOR MORE INFORMATION
Morten Frank Pedersen
Head of Public Policy, Denmark & Nordic
Takeda Pharma
morten.pedersen@takeda.com

