Access of Rare Disease Treatments in the Nordics

HPR145

Sami Gabbouj MSc[1], Saku Torvinen PhD[1], Ulrikke Voss PhD[2], Tero Ylisaukko-oja PhD[1]

[1.] MedEngine Oy, 00130 Helsinki, Finland; [2.] MedEngine Aps, 1157 Copenhagen, Denmark

Objective

Rare disease treatments are among the emerging therapies most frequently seeking market access.[1] The Nordic countries of **Denmark**, **Finland**, **Norway**, and **Sweden** have established access processes for medicines.[2] Rare disease treatments are characterized by a scarcity of robust clinical data and high costs.[3] This presents a challenge for Health Technology Assessment (HTA).

Aim

This study aimed to identify challenges and opportunities in access processes and funding for rare disease medicines as compared to non-rare disease medicines in the Nordics.

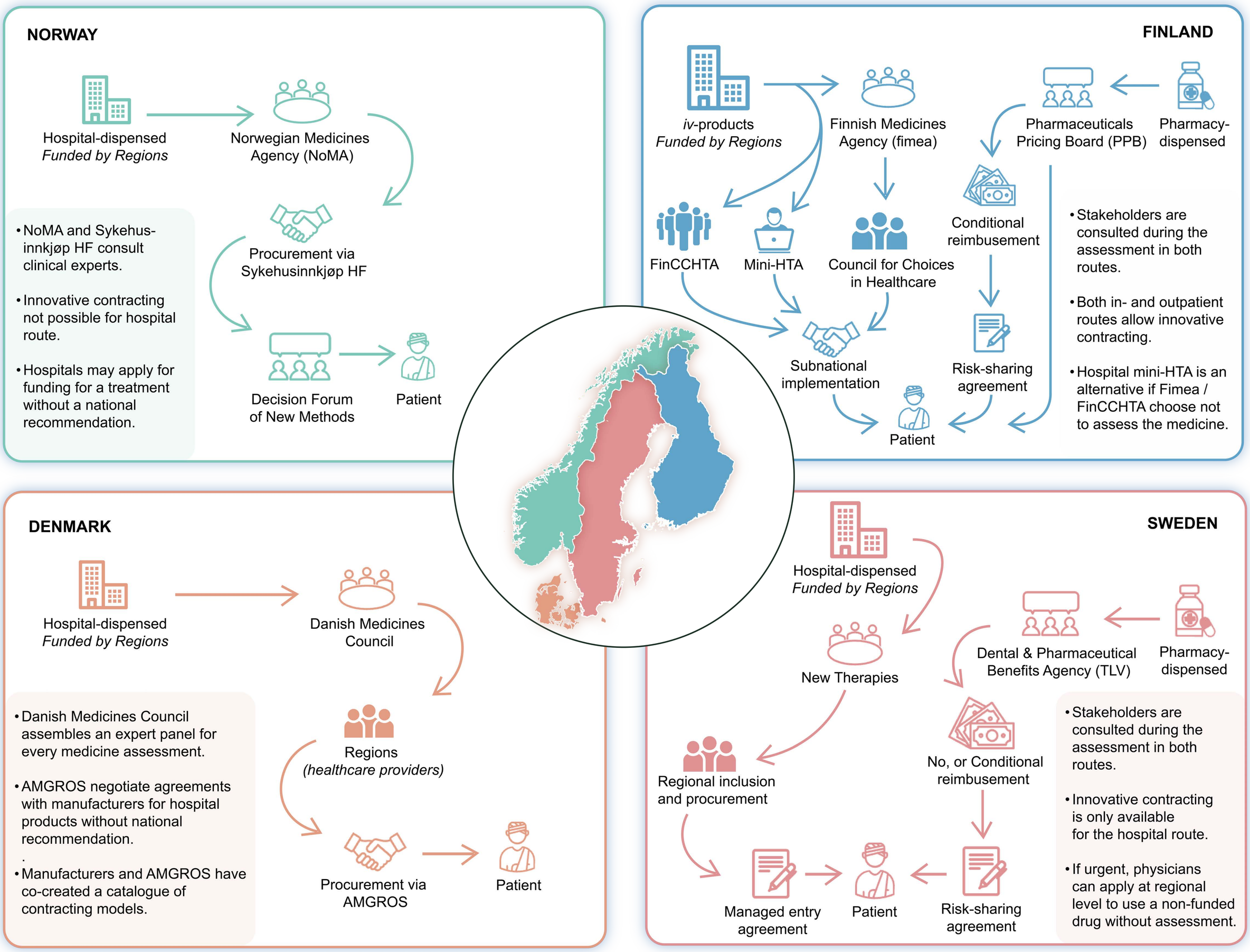
Methods

Analyses of country-specific access procedures were based on 1) a literature review of scientific publications and publicly available reports and 2) semi-structured interviews of 1–2 experts per country working with rare disease treatment access in the industry or regulatory bodies. The questionnaire assessed themes within HTA, pricing and reimbursement, access initiatives, and future developments.

Results

Rare disease medicines are not formally given special consideration in HTAs in any Nordic country. However, higher willingness to pay and tolerance for uncertainty in clinical and health economic evidence may be applied on a case-by-case basis.

Diagrams outline the access process for rare disease medicines in each of the Nordic countries.



Conclusion

The Nordic countries are an interesting market to explore for launching rare disease treatments. This is highlighted by similar access requirements for medicines, a strengthening of cross-country collaboration, and a willingness to establish new access solutions to rare disease medicines. These include innovative pricing schemes which have been developed to tackle high treatment costs and lack of robust clinical data.

References

1. Orphan Medicinal Product Designation: Overview 2000–2022. European Medicines Agency. 2023.
2. Nordic Rare Disease Summit – An Assessment of Alignment of P&R Systems with ORPH-VAL Principles. Dolon. 2021.
3. Policy statement on rare diseases in the Nordic countries. Nordic Rare Disease Summit. 2023.

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Conflict of interest
Authors declare no conflicts of interest

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