Endo Compass

Research roadmap for better hormone health

The EndoCompass Research Roadmap

Policy priorities for better hormone health in Europe

Finding cures for rare endocrine diseases

Why this matters for Europe:

- Hormones matter at every stage of life, from early development to older age.
- Some of Europe's most costly health challenges are linked to endocrine disruption, such as diabetes, obesity, thyroid disease, cancer, osteoporosis and infertility, and more than 440 rare endocrine diseases.
- Most Europeans will be affected by an endocrine-related condition in their lifetime, weakening our capacity to live, work and age well.
- Endocrine science is an investment in Europe's future.

ESPE European Society for Paediatric Endocrinology

European Society of Endocrinology

This document is an excerpt from *The EndoCompass Research Roadmap – Policy Priorities for Better Hormone Health in Europe*. For more information, please contact the European Society of Endocrinology at info@ese-hormones.org or the European Society for Paediatric Endocrinology at secretariat@eurospe.org

Finding cures for rare endocrine diseases

Why this matters

It is not uncommon to have a rare disease. Up to 36 million people in the European Union live with a rare condition.[2] There are more than 440 rare endocrine conditions that affect small numbers individually, but collectively represent a healthcare burden comparable to a major common condition like cancer or diabetes.[3] These conditions bring specific challenges that call for coordinated action at European level.

Small patient populations and scattered clinical experience make rare endocrine conditions harder to study and manage. Patients face diagnostic delays and fragmented services. Most rare endocrine diseases are lifelong and start in childhood, requiring many years of complex and changing care. As prevalence increases and patients live longer, the effect on individuals, families and public health systems means these conditions should be understood as a high-impact disease group.

Many rare diseases cannot be prevented or improved through lifestyle changes, leaving patients reliant on advances in diagnosis and treatment. However, for around 95% of rare diseases, effective treatment options do not yet exist.[23] Incentives for commercial drug development are limited, even though the costs for individual patients and society are high.

Progress depends on cross-border collaboration and harmonised data-sharing through registries. The EndoCompass Research Roadmap describes what is needed for a well-structured rare disease ecosystem in Europe, supported by stronger public–private partnerships and investment in basic science, digital infrastructure and artificial intelligence.

Such an approach would align with calls for a European action plan on rare diseases (under discussion at the time of writing).[24] It would also maximise the wider benefits of rare disease research, where breakthroughs in diagnostics can be translated to other more common disease groups and strengthen Europe's competitiveness in biomedical research.

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What research is needed?

Research on rare endocrine diseases has traditionally focused on paediatric populations, reflecting the fact that many conditions that limit lifespan begin in early life. Growing recognition of rare diseases that present or are acquired in adulthood, combined with improved survival into later life, means research must now take a full life-course approach. This includes understanding disease progression and improving the transition from paediatric to adult care.

The EndoCompass Research Roadmap identifies the following priority focus areas for research into rare endocrine conditions:

1. Diagnostic research readiness
Research should focus on building Europe's capacity to diagnose rare endocrine diseases earlier and more accurately. This should include the use of standardised, secure datasets to enable reliable data sharing between different research facilities in different countries. Investing in pipelines for coordinated genomic research and innovation in genomic diagnostics will help reduce diagnostic delays and uncertainty.

2. Clinical trial readiness
New research approaches are needed to run clinical trials involving small, dispersed patient groups. Priorities include multinational patient inclusion, cross-border collaboration and disease progression modelling to support innovative trial designs.

3. Clinical outcome assessment
Clinicians need better evidence on how to define, measure and work towards the outcomes that matter to patients. Patient-reported outcome measures are central to this, along with understanding the real socioeconomic impact of rare endocrine diseases. This also calls for developing and implementing the right digital tools and disease-specific clinical outcome assessment tools to guide clinical decisions.

4. Innovative therapies and EU-wide availability
Research should accelerate the development of innovative therapies for rare endocrine diseases by strengthening the translational pathway from basic discovery to 'proof-of-concept' and clinical application. This includes building a more supportive ecosystem around the development and regulation of orphan drugs and new indications for known drugs.

Governments also need to ensure more efficient and equitable access to new treatments and expert clinical care. This should include mapping advanced therapy medicinal products and analysing treatment availability in EU Member States.

5. Mapping patient pathways
Finally, defining disease-specific patient journeys and referral routes for rare endocrine care will show how patients currently move through health systems, where variation exists and where future improvements could be tested, including for those with lifelong endocrine disorders.

For more information on proposed research priorities in this area, please refer to EndoCompass project: rare diseases in endocrinology.

Investing in hormone health is one of the most effective ways for Europe to prevent and manage disease, support healthy ageing and sustain its health systems and economy.

The endocrine community calls on policymakers to deliver the EndoCompass recommendations and ensure a healthier, more resilient Europe.

EndoCompass

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To discuss the recommendations in this paper or related policy engagement activities, please contact ESE or ESPE emailing: **info@ese-hormones.org** or **secretariat@eurospe.org**

EndoCompass Research Roadmap – Directions for the Future of Endocrine Science was developed as a joint initiative of the European Society of Endocrinology and the European Society for Paediatric Endocrinology. It is published as an open-access supplement in the *European Journal of Endocrinology* and the *Hormone Research in Paediatrics* journal.

Find out more about the EndoCompass Research Roadmap : ese-hormones.org/endocompass.

This document is an extract from *The EndoCompass Research Roadmap – Policy Priorities for Better Hormone Health in Europe*, a wider set of policy recommendations for improving hormone health across Europe. Read the full paper: ese-hormones.org/endocompass-policy-paper

References*

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*Reference numbers follow the numbering used in the full paper.

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