

Meeting Report / Position Paper
screen4rare – MEP Alliance for Rare Disease Newborn Screening

Newborn Screening for Rare Diseases: How can the EU lead on the global stage?

European Parliament, Brussels – 23 September 2025

The high-level event organised by **screen4rare** (S4R) and the **MEP Alliance for Rare Disease Newborn Screening** on 23 September 2025 in the European Parliament, hosted by **MEP Tomislav Sokol (EPP, Croatia)**, confirmed a strong and united call: the EU must take decisive steps to lead on Newborn Screening (NBS) for Rare Diseases.

MEPs highlighted the central role NBS should play in a renewed European strategy on Rare Diseases and in health innovation. **MEP Tomislav Sokol (EPP, Croatia)** called for an updated and comprehensive EU Rare Disease Action Plan with clear benchmarks, strong support for orphan medicines and ERNs, and common EU guidelines on NBS. **MEP Sirpa Pietikäinen (EPP, Finland)** urged a truly European approach to health, ensuring data-driven research and equity across Member States. **MEP Billy Kelleher (Renew, Ireland)** stressed that while ethical issues around genomics must be addressed, lives are at stake and newborns cannot wait – urging the EU to set guidelines, provide benchmarking, and accelerate progress through a new EU Action Plan on Rare Diseases. Furthermore, leveraging the EU Life Sciences Strategy and the upcoming European Biotech Act, ensuring that NBS is prioritised within these flagship initiatives, will be essential to strengthen Europe’s leadership in health innovation and Rare Diseases policy.

NBS is no longer just a technical issue but a strategic test for Europe’s Rare Disease and health innovation agenda. S4R members **Johan Prevot (IPOPI)** and **Peter Schielen (ISNS)** underlined the urgency of addressing disparities across Member States and the opportunity for the EU to fill the global leadership gap left by the US elimination of its Advisory Committee on Heritable Disorders in Newborns and Children. **Prof. Maurizio Scarpa (MetabERN)** showcased the European Reference Networks (ERNs) as a flagship EU achievement, demonstrating how their expertise and infrastructure can drive cooperation and knowledge-sharing on NBS. **Prof. Jim Bonham (ISNS)** presented the S4R proposal for an ERN advice and best practice working group on NBS – recently welcomed by the ERN Board of Member States – as a trusted mechanism to guide EU countries, promote equity, and ensure evidence-based implementation across the EU.

The scientific frontier was addressed by **Prof. Lennart Hammarström**, world-leading expert in genomic NBS from the Karolinska Institutet. He highlighted the transformative potential of genomics to revolutionise early diagnosis and treatment for Rare Diseases, provided it is introduced with the right safeguards and governance – showing how the EU can turn scientific excellence into public health leadership.

Strong alignment emerged across all interventions: **NBS for Rare Diseases is a European priority that can no longer be left to fragmented national approaches. The EU has the instruments, expertise, and responsibility to drive harmonisation, embed genomics safely, strengthen ERNs, and turn best practice into common practice.** Europe must act now to unite around a common strategy, equipping Member States with the guidance needed to ensure every child, wherever born, has equal access to life-saving early detection – a defining opportunity to advance health equity and European leadership in Rare Diseases and innovation.

screen4rare’s meeting highlighted three main conclusions:

- While NBS must be embedded within the EU Life Sciences Strategy and the upcoming European Biotech Act, the EU should adopt an updated Rare Disease Action Plan with clear targets.
- The newly established ERN advice and best practice working group on NBS will be essential to guide Member States, promote equity, and build coherence.
- Genomic NBS, introduced with the right safeguards and governance, offers a unique opportunity for Europe to lead globally – scientifically, politically, and ethically – turning innovation into equitable public health impact.

