



Call to Action
Spinal muscular atrophy:
Screen at birth, save lives

Call to Action, v1, 26 March 2021

European Alliance for Newborn Screening in SMA

Call to Action - Recommendations by the Alliance Steering Committee

This Call to Action is initiated by the European Alliance for Newborn Screening for Spinal Muscular Atrophy, a multi-stakeholder initiative under the leadership of SMA Europe e.V.

“There is no more time to waste for babies born with SMA - newborn screening programmes for SMA in all European countries no later than 2025”

The **European Alliance for Newborn Screening in SMA’s** aspirations are aligned with the advocacy goals of other key ecosystem stakeholders in relation to newborn screening;

- Considering the UN convention on the Rights of the Child ratified by all European Countries mandating governments to secure optimal health care for children,
- Recognizing the European Union’s commitment to achieve Universal Health Coverage in its territory by 2030,
- Acknowledging the initiatives for early detection of severe inherited diseases brought forward by EURORDIS- Rare Diseases Europe (1) and the call-to-action of the Screen4Rare initiative (2) and other academic and patient-led multi-stakeholder consortia,
- Considering that newborn screening programmes in Europe screen for a vastly different number of diseases depending on the country and sometimes region (ranging from 2-48 diseases),
- Emphasizing the overwhelming evidence that confirms that SMA meets the WHO criteria to be included in newborn screening programmes in order to ensure an early diagnosis and an appropriate treatment that can prevent or at least significantly delay severe impairment and/or early death in infancy,
- Strongly opposing the inequality of access of SMA newborn screening for babies born in Europe,
- Appreciating the fact that this lack of access to newborn screening for SMA contradicts the policy of the European Union to ensure appropriate health care to children as one of the most basic rights children can enjoy and
- Expressing our willingness to partner and join forces with all relevant stakeholders to secure better health care for children born with SMA in Europe now,

We hereby urge policymakers across EU to take action on realizing the aspirations of **the European Alliance for Newborn Screening in Spinal Muscular Atrophy (SMA)**;

Call to Action for policy makers at the European level

1. Coordinate the exchange of knowledge and best practices on newborn screening in SMA and other eligible rare diseases, including learnings from ongoing pilots. While we appreciate the responsibility of the EU Member states in ensuring sufficient access to health care, we interpret the principle of subsidiarity with regard to health care in a way that the EU has a strong remit in fostering equal access to health care across the EU.
2. As newborn screening pilot programmes for SMA in a range of member states are finished / ongoing / planned including Belgium, Italy, Germany, Spain, France and the United Kingdom, we ask to support both financially and organisationally the meta-analysis of the results of these programmes and the identification of key learnings with regard to implementation in standard newborn screening programmes across Europe.
3. As best practice sharing can help member states to implement newborn screening for SMA by learning both from other Member States and non-EU countries, we ask the European Commission to gather key learnings including but not limited to
 - a. gathering evidence and natural history data on efficacy from pilot studies on newborn screening for SMA
 - b. identifying and agreeing upon criteria and mechanisms for expanding the number of diseases to be included in screening panels
 - c. implementation strategies for expanding existing newborn screening programmes
 - d. suitable screening procedures
 - e. requirements for education and training of professionals and communication with families and citizens.
4. Newborn screening in rare diseases, including but not limited to SMA, is a key instrument to ensure equal access to diagnosis and subsequent appropriate therapy for children with rare diseases in Europe. We therefore ask the European Commission and other stakeholders at the EU level to monitor and support all measures helping to improve newborn screening for SMA.
5. We also encourage EU institutions to recommend adding SMA to a list of recommended diseases to screen for at birth and support countries in the implementation of expanding newborn screening.

Call to Action for [policy makers at the national level](#)

1. We urge national competent authorities to include SMA in the list of diseases eligible to be included in national and/or regional newborn screening programmes without any further delay.
Based on growing evidence, SMA clearly meets the WHO criteria to be included in the newborn screening programmes. Early diagnosis and treatment initiation can prevent early death in infancy and significantly delay severe impairment in later stages. Identifying and treating SMA early on provides a better outcome for children affected by SMA. Almost four years after the first new generation treatment for SMA became available, patients in the vast majority of European countries still lack access to timely diagnosis through newborn screening.
2. We further call on national governments and parliaments to ensure sufficient funding of newborn screening for SMA including an appropriate, fast and sustainable implementation.
3. We ask national competent authorities to draw on the experiences from the ongoing pilot programmes in other European countries and to make use of the support provided by the European Union in reducing access barriers to newborn screening for SMA.
4. National SMA patient organisations play a crucial role in providing patient insights, family support and public guidance during the implementation of newborn screening in SMA. We strongly suggest national parliaments to support their advocacy efforts for newborn screening to include SMA.

The **European Alliance for Newborn Screening in Spinal Muscular Atrophy** demands to national governments and authorities in Europe to immediately include a test for spinal muscular atrophy for all newborn children in national newborn screening programmes. There is no more time to waste for babies born with SMA to start adequate treatment.

The Alliance therefore calls on all decision-makers in Europe to implement this essential health service in all European countries without any further delay.