Spinal muscular atrophy in the UK: the human toll of slow decisions

Spinal muscular atrophy (SMA) is a recessive condition that affects globally one in 14 800 newborns.[1] Infants with SMA type 1 do not acquire motor milestones and rarely survive beyond the first year of life. Children with SMA type 2 are never able to walk independently, and most individuals with SMA type 3 lose the ability to walk before the age of 30 years. Since 2017, three treatments—nusinersen, risdiplam, and onasemnogene abeparvovec—have been approved by regulatory authorities in the USA and Europe.[2] These treatments have shown much improved efficacy when delivered early in life,[1] and have led to the initiation of several newborn screening programmes for SMA since 2018.[3] In 2018, a request to consider a newborn screening programme for SMA was rejected by the UK National Screening Committee (NSC) on the rationale that there was insufficient evidence to suggest that the intervention would do more good than harm. This request is currently under re-evaluation. Both the initial submission and the current re-evaluation have been supported unanimously by physicians and patient associations. A request for an earlier re-evaluation based on compelling international peer-reviewed evidence was unsuccessful.

Between Jan 1, 2019, and Nov 1, 2024, 507 patients with SMA (213 [42%] with SMA type 1, 188 [37%] with SMA type 2, and 106 [21%] with SMA type 3) were registered in the SMA REACH UK database. The mean age at diagnosis was 0·5 years (SD 0·4) for those with SMA type 1, 1·6 years (SD 0·8) for those with SMA type 2, and 2·9 years (SD 1·7) for those with SMA type 3. As of data cutoff (Nov 1, 2024), 16 (3%) of 507 patients had died. None of those with SMA type 1 had developed the ability to walk independently. 129 (65%) of 198 individuals with SMA type 1 who were alive were on ventilatory support and 106 (54%) required nutritional support via tube feeding. These data contrast substantially with those from other European countries such as Germany or Belgium, where newborn screening has been implemented in a timely manner.[3,4] During the period Jan 1, 2019, to Nov 1, 2024, in southern Belgium, where newborn screening was in place,[4] none of the 20 infants who were born with SMA died, none were on ventilatory or nutritional support, and all 16 children with SMA who were older than 2 years were ambulatory (Servais L, Dangouloff T, unpublished).

The 2018 decision to refuse an SMA newborn screening programme in the UK, despite the support of the entire national SMA community and in disregard of international evidence, resulted in substantially different outcomes for children born in the UK compared with those born in many other countries where newborn screening is available. The annual cost of rehabilitation and management, in addition to the novel SMA treatment costs for each symptomatic patient with SMA type 1, has been determined to be £74 415, compared with £24 671 for similar patients identified through newborn screening programmes.[5]

We propose that expert opinion[6] and international evidence[1,3] should be more thoroughly integrated into the decision-making process of NSCs. Furthermore, the decision of independent bodies such as the NSC should be subject to scrutiny by the Ministry of Health, given the substantial effect of failing in the duty of care. Evidence provided by national and international researchers,[1,3,6] including investigator-initiated screening pilots,[4,7] health economic modelling,[8] and expert consensus,[6,9] should be given much more careful consideration.

TD and LS provided and analysed Belgian data. FM, MS, and GB provided and analysed UK data. LS drafted the initial manuscript. LS and MS accessed and verified the underlying study data. All authors had full access to the data in the study and had final responsibility for the decision to submit for publication. LS has received consultancy fees and grants from Roche, Biogen, Novartis, Scholar Rock, BioHaven, and Zentech (Lacar). TD has received lecture fees from Biogen and Roche. MS has received consultancy fees from Roche, Biogen, and Novartis; and honoraria and support for attending meetings from Novartis and Roche. GB has received grants from Sarepta, Roche, Novartis, Pfizer, Italfarmaco, and Santhera; consulting fees from Sarepta, Entrada Therapeutics, Pfizer, Biogen, Novartis, and Roche; and honoraria and support for attending meetings from Novartis and Roche. FM has received consultancy fees from and received fees for participation in symposia for Roche, Biogen, and Novartis; has received honoraria from Biogen; has received support for attending meetings from Roche; and is on advisory board for Roche and Biogen. De-identified participant data can be made available upon request to the corresponding author by qualified investigators. The newborn screening pilot in Belgium was funded by AveXis, Biogen, Roche, the Association Belge contre les Maladies neuro- Musculaires, Minister's Office Alda GREOLI (Wallonia-Brussels Community), and donations from individuals. SMA REACH (SMA REsearch And Clinical Hub) is funded by grants from Novartis, Biogen, and Roche. There was no funding for this Correspondence.

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For more on the **request to fund newborn screening in the UK** see https://petition.parliament.uk/archived/petitions/588447

For the **SMA Reach UK website** see https://smareachuk.org/

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Gender removal by fiat: impacts of new Trump administration edicts

We write to alert *Lancet* readers that, as of Feb 1, 2025, all US research and websites published by federal scientists and agencies are now subject to new edicts issued by the Trump administration[1,2] that are contrary to established norms, and contradict the guidelines of *The Lancet*[3] and the International Committee of Medical Journal Editors (ICMJE).[4]

For example, all US Centers for Disease Control and Prevention researchers have been instructed to remove references to, or mentions of, the following terms: "Gender, transgender, pregnant person, pregnant people, LGBT, transsexual, non-binary, nonbinary, assigned male at birth, assigned female at birth, biologically male, biologically female."[2] This instruction from the Trump administration, "to retract or pause the publication of any research manuscript under consideration by any medical or scientific journal, not merely its own internal periodicals",[2] diametrically opposes the instructions provided by *The Lancet*[3] and the ICMJE[4] (appendix).

See Online for appendix

Even more expansive edicts have already been applied to US Government websites, resulting in numerous websites and datasets being taken down that use not only these terms, but also other terms such as health equity, climate change, and environmental justice.[5] In this moment of unprecedented political interference in the conduct, publication, and dissemination of federally supported science in the USA, we ask that everyone in the global scientific community be attentive to these threats to accuracy, and to possible suppression of scientific evidence involving US federal agencies and consequently authors employed by these agencies.

We declare no competing interests.

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Angola's cholera outbreak: a wake-up call for global action

Angola's Ministry of Health has released alarming statistics regarding the ongoing cholera outbreak,[1,2] highlighting a severe public health crisis that warrants immediate international attention. By Feb 3, 2025, Angola had recorded 1888 cases and 65 fatalities since the outbreak began. Over the past 24 h alone, 178 new cases were reported across multiple provinces, with a concentration in Luanda. Epidemiologically, children aged 2–9 years are the most affected, with 294 cases and ten fatalities, followed by those aged 10–14 years, with 250 cases and five deaths.[1] Women and girls account for 47% of cases, possibly due to differences in access to sanitation or health care.[1] This susceptibility underscores the urgent need for targeted interventions to protect younger populations. Geographically, the province of Luanda remains the epicentre, with 1062 cases and 38 fatalities, concentrated in densely populated areas. Other provinces, including the newly established Icolo e Bengo (n=189) and Bengo (n=105) provinces, have also been substantially affected. The detection of cases with links to Luanda in distant regions such as Lubango suggests the potential for further geographical spread.[1,2]

Cholera's resurgence in Angola and sub-Saharan Africa reflects broader systemic issues, including inadequate access to clean water, poor sanitation, and overstretched health-care systems.[3,4] Although commendable efforts are underway, system-wide measures are urgently needed to control the outbreak. Recently, Angola's Ministry of Health released the updated *Manual for the Prevention and Control of Cholera and Other Acute Diarrheal Diseases*,[5] which provides standardised tools to address cholera and other diarrhoeal diseases, emphasising prevention, early detection, and outbreak response.[5] The manual highlights community education and collaboration between governmental and non-governmental entities as essential strategies for reducing transmission, improving early case detection, and ensuring effective outbreak response through coordinated interventions.

Addressing Angola's cholera outbreak requires enhanced surveillance systems to detect and contain cases quickly. For example, deploying