Help Us Challenge the UK NSC's Decision on MLD Newborn Screening

Dear Parents, Families and Supporters,

This campaign is being led by three patient organisations working tirelessly to protect and support children with Metachromatic Leukodystrophy (MLD): **ArchAngel MLD Trust**, **MLD Support Association UK**, and the **MPS Society**.

Together, we are calling for urgent action to help challenge the UK National Screening Committee's decision **not to add** MLD to the newborn screening programme, despite the availability of a life-saving treatment. We are bringing together families, clinicians, and experts who understand the devastating impact of delayed diagnosis, and are united in pushing for change.

We urgently need your help.

MLD is not currently included in the UK newborn screening programme, and this is the first time it has been formally reviewed by the UK National Screening Committee (UK NSC).

The 2025 evidence review looked at whether screening newborns for MLD is accurate, effective, and offers value for money.

The review found that important questions remain especially about which test is best and whether early diagnosis through screening leads to better outcomes.

Because of this, the UK NSC is minded not to add MLD to the screening panel at this time, but will keep the condition under review (review cycle of 3-4years) as new evidence from research and international programmes becomes available.

This delay will mean more children will die or suffer irreversible decline before diagnosis, despite an effective treatment (Libmeldy) now being available.

As parents and families, your voice is powerful. We are asking you to contact the UK NSC copying in your MP to challenge this decision and demand an urgent reassessment of the available evidence.

The link to the NSC review can be found here… **PLEASE NOTE THE DEADLINE OF 5TH AUGUST 2025**

https://view-health-screening-recommendations.service.gov.uk/metachromatic-leukodystrophy/

The link to find your MP can be found here…

https://members.parliament.uk/FindYourMP

Here's why you should act now:

The NSC's evidence review is deeply flawed.

- It ignored or rejected key clinical trial data published in world-leading journals
- It excluded real-world evidence from UK families, clinicians, and patient groups including two published caregiver studies that NICE itself called "among the most robust they had ever seen"
- It did not involve any clinicians, laboratories, or patient organisations with experience in MLD when reviewing the evidence
- It ignored the fact that children treated early are doing well, while those diagnosed late suffer and die young

Why this decision matters:

- Children are dying or being denied life-saving treatment because their disease is found too late.
- We now have an effective gene therapy (Libmeldy) that can stop the disease before it starts, but it only works if given before symptoms begin.
- Newborn screening is the only way to find these children in time.
- Other countries are moving ahead with MLD screening, while the UK is falling behind.

Since the treatment was approved in 2022:

- Only 6 children in the UK have been treated, because they were diagnosed early
- 30 children have been turned away, because they were diagnosed too late

What can you do?

We're asking all parents, families, and supporters to take action:

1.Contact the UK NSC and copy in your MP. We have provided a template letter for you to use and adapt

  - Tell them MLD must be added to newborn screening

- Let them know that delays are costing children their lives

2. Share your voice and your story.
Even if your family hasn't been directly affected, speaking up helps us protect others.

3. Consent for the patient organisations named above to share your story and feedback.

Consent to Share Story and Feedback

I give my permission for ArchAngel MLD Trust, MLD Support Association UK and The MPS Society to share my story and/or feedback as part of their ongoing campaign to improve access to newborn screening for Metachromatic Leukodystrophy (MLD). I understand that my contribution may be used to support advocacy efforts, raise awareness, and influence policy decisions.

☐ I consent to my story and/or feedback being shared
☐ I understand that my information may be used in written or verbal submissions.
☐ I understand that I can withdraw my consent at any time by contacting Georgina Morton at contact@ArchAngel.org.uk

Name……

Signed…….

Date……..

No parent should ever hear that their child could have been treated if only the disease had been found earlier.

Please help us stop this from happening to more families.

Thank you

ArchAngel MLD Trust MLD Support Association UK The MPS Society

Template letter

To the UK National Screening Committee,

I am writing to express my deep concern and disappointment regarding the recent decision not to include Metachromatic Leukodystrophy (MLD) in the UK's newborn screening programme.

As a (parent / family member) I cannot understand why we are not screening for a fatal childhood disease when there is a proven treatment but only if the disease is caught early, before symptoms appear.

I urge the NSC to consider the following questions:

- Why are we not screening for a fatal childhood disease when there is a treatment that works if caught pre-symptomatically?
- How many more children have to miss out on treatment before MLD is added to the newborn screening panel?
- Why did the NSC ignore real-world evidence, families, and UK clinical experts in their decision?
- Why is the UK ignoring the advice of doctors, scientists, and families who know MLD best?

As a parent, I would want to know if my baby had a treatable condition like MLD before it was too late.

Every child deserves the chance to live a full life. Without newborn screening for MLD, that chance is taken away.

We have the treatment. The only missing piece is early diagnosis. So why isn't the UK using the tools we already have? Other countries are already screening for MLD why is the UK choosing not to protect our children?

No parent should ever be told that their child could have been saved if only they'd been screened at birth. Yet we know that at least 30 children in the UK have already been denied treatment because their diagnosis came too late. This is preventable.

(Optional: Please share your personal story, thoughts, or reasons why you believe MLD should be added to newborn screening)

I strongly urge the NSC to reconsider its position and meet with clinical experts, families, and patient organisations working with MLD. This is a rare opportunity to **prevent suffering and save lives** but only if we act now

Yours sincerely