

## Public consultation on the proposed operational framework for individualised genetic therapies: NHS England Draft scope for engagement

British Society for Immunology Clinical Immunology Professional Network (BSI-CIPN)  
response

*January 2026*

***Do you have any comments on the purpose and remit of the operational framework as described in the scope?***

The British Society of Immunology Clinical Immunology Professionals Network (BSI-CIPN) is a network of healthcare professionals working within clinical immunology, including adult and paediatric physicians, specialist nurses, pharmacists and healthcare scientists. Patients with inborn errors of immunity (IEIs, rare genetic disorders of immunity, previously known as primary immunodeficiencies) are treated, managed and supported by professionals within this network.

The clinical immunology community are strongly supportive of this initiative. Individualised genetic therapies offer enormous hope to patients with inborn errors of immunity. These disorders are all ultra-rare and have limited standard of care treatment options, often associated with high levels of toxicity.

- Patients with inborn errors of immunity have been at the forefront of research into genomic diagnostic and individualised genetic therapies, and have volunteered as research subjects during early pioneering studies in these areas. As these technologies are rolled out to other rare diseases it is important that patients with IEIs are not marginalised or forgotten.
- The development of an operational framework should have robust input from patient advocacy groups and charities representing patients/carers with rare diseases. It is essential that the patient voice of those living with these conditions, often with a poor long term prognosis, is heard and incorporated into any service delivery framework. This is particularly important when assessing the risk-benefit balance of providing highly innovative therapies on a compassionate basis.
- The draft scope for engagement refers only to germline genetic disorders. We would emphasise the importance of ring-fencing treatment for these patients, particularly as individualised genetic therapies for malignancies are rapidly evolving. With the development of these new cancer treatments, it is essential that patients with rare diseases remain prioritised.
- We would encourage this operational framework to facilitate flexibility around the mode of delivery of individualised genetic therapies. Genetic therapies have been delivered intravenously, topically, intrathecally and directly in to target tissues, and it will be important that any operational framework allows the flexibility to respond to changes in treatment delivery as clinically appropriate or based on international experience.

*Is the definition of “individualised genetic therapies” clear and appropriate for this framework?* Yes

*Are there any terms or concepts in the scope that are unclear or require further clarification?* No

*Are there any additional topics that should be included in the scope that are not already listed?*

Full service evaluation for delivering individualised genetic therapies must be stipulated within this framework. For many individualised genetic therapies, existing clinical services will be unable to deliver these therapies equitably within the existing service framework. Learning from the challenges highlighted by the compassionate availability of givinostat to treat children with Duchenne Muscular Dystrophy in 2025 is essential. In this situation a genetic therapy was provided free of charge by a commercial company closely liaising with patient advocacy groups, however national clinical services were unable to deliver this treatment equitably without significant clinical service expansion.

- Many individualised genetic therapies will change the long term full economic healthcare costs associated with specific diseases. The initial costs of delivering these therapies may be higher, however longer-term costs are likely to be reduced. When considering the economic feasibility of delivering individualised genetic therapies, an offset care cost model should be considered, allowing upfront increased costs to be balanced against long term savings.
- The operational framework should incorporate compulsory real world data collection around the use of individualised genetic therapies. This should include formal health economic and social sciences data as well as clinical outcomes and toxicities. Mandatory long-term outcome, toxicity data and quality of life should be collected.
- Guidance and interaction with the medicine regulator (Medicines and Healthcare Products Regulatory Agency) is essential during the development of this operational framework. Specifically.
  - o Approval for a proportionate risk model to delivering these therapies
  - o Agreement that rare patient cohorts treated on a compassionate basis can have outcome and safety data published with MHRA approval, to improve the international experience and understanding of these treatments.

*We list what the operational framework will cover on page 4. Do you think any of these topics should be removed and if so, why?* No

*What the operational framework won't cover*

*The scope on Page 4 outlines the exclusions as follows:*

- *Clinical decision making on who might be suitable to receive an individualised genetic therapy*
- *Funding strategy, prioritisation and process, including how an individualised genetic therapy and any associated service costs might be funded in the future*
- *Identifying treatments for people where no individualised genetic therapy currently exists*
- *Study approvals, including approach to patient consent / assent and research ethics*
- *Clinical evidence interpretation and evaluation*
- *Regulatory requirements and methodology for individualised therapies*

- *Health technology assessment requirements and methodology for individualised therapies*
- *Commercial strategy and pricing / supply negotiation mechanisms for individualised therapies*

*Are there any exclusions you think we should add?* No

*Are there any exclusions you think we should remove and if so, why?*

Point 17 Regulatory requirements and methodology for individualised therapies should be supported through this framework. This should be included as it will be critical how compassionate use of individualised genetic therapies are implemented.

*In which medical conditions do you expect individualised genetic therapies to be produced in the next 5 years?*

- Many IELs
- Wiskott Aldrich syndrome
- Leucocyte adhesion defect
- AR chronic granulomatous disease
- Activated PI3K Delta syndrome
- CTLA haploinsufficiency
- IPEX syndrome
- XMEM
- X-linked agammaglobulinaemia
- X-linked lymphoproliferative disorder

*We outline how potentially eligible patients might best be identified on Page 4. Do you have any comments on this?* Any guidance around patient eligibility and access to individualised genetic therapies should maximise equity of access and reflect the phenotypic variability of patients with rare diseases. In commercially sponsored clinical trials eligibility criteria for recruitment are usually strict and specific to the marketing authorisation aim of the commercial company. Where treatment funding is coming from philanthropic / academic / not-for-profit organisations (compassionate funding) it is important to establish patient-focused eligibility criteria which reflect the needs of patients and their physicians.

*Do you believe there are any additional risks or unintended consequences that should be considered as we develop this framework?* Yes

*Are there any particular considerations regarding protected characteristics (Equality Act 2010) that should be taken account of in the operations framework?*

- Treatment with individualised genetic therapies risk the development of a “postcode lottery” for treatment, and consideration of specialist centres should be made in conjunction with specialist clinical networks. As treatment centres are likely to be highly centralised, other economic factors (such as travel costs, accommodation cost) may drive inequity of access to treatment. Awareness of different services required for treating paediatric and adult patients should be recognised.
- Also points made above around patient eligibility to ensure criteria are not too narrow to exclude patients economic status of patients is also a potential barrier

*Are there further considerations that could strengthen equity of access to individualised genetic therapies?*

No answer

*Do you have any additional comments or suggestions to improve the scope?*

No answer