



Consultation Response: MHRA Draft Rare Disease Therapies Regulatory Framework

About you

Which best applies to you?

I am responding on behalf of an organisation.

My organisation is a

Professional Body.

Please tell us the geographical area(s) your organisation covers

England; Scotland; Wales.

Name of organisation

Royal College of Pharmacy.

The first three questions are about the overall aim of the framework and the involvement of patient views

1. In your view, is it desirable for more pharmaceutical treatments for rare diseases to be brought into a regulated pathway rather than used 'off-label' as many are at present? Please explain your answer.

Yes. The Royal College of Pharmacy supports, in principle, the aim of bringing more pharmaceutical treatments for rare diseases into an appropriate regulated pathway, where this can be achieved without creating unnecessary barriers to timely access.

Off-label use can be clinically necessary, particularly where there is high unmet need and limited licensed treatment availability. However, reliance on off-label use may result in variable evidence standards, inconsistent patient information, uncertainty around dosing and monitoring, and less structured pharmacovigilance. A regulated pathway has the potential to provide greater clarity on indication, quality, safety monitoring, benefit-risk assessment, prescribing responsibilities, patient communication and data collection.

This is particularly important given that rare diseases are collectively common, affecting around 1 in 17 people in the UK, while only around 5% of rare conditions have an approved and effective treatment.^{3,4} The proposed framework recognises that conventional development pathways are often not feasible because of small populations, limited natural history data, lack of validated endpoints and urgent unmet need.^{1,2}

However, implementation should preserve clinical judgement and avoid unintentionally disrupting existing off-label access where this is supported by sufficient evidence, specialist consensus and established clinical practice. The framework should be positioned as a proportionate regulatory route that strengthens oversight, transparency and evidence generation, particularly where evidence is limited or important safety uncertainties remain, rather than as a mechanism that automatically displaces appropriate off-label use. The nature and intensity of regulatory requirements should reflect

whether the therapy is a new active substance, an advanced or individualised therapy, or a repurposed medicine with substantial prior clinical exposure.

2. The consultation sets out challenges associated with the development and regulation of treatments for rare diseases. Please identify any other challenges you are aware of that have not been considered or elaborate on those already identified. Optional.

The framework identifies many of the key scientific and regulatory challenges. We would encourage further consideration of the following implementation challenges.

First, pharmacy and medicines optimisation infrastructure will be critical. Rare disease therapies, particularly advanced therapies and individualised medicines, may require complex procurement, storage, preparation, administration, monitoring and traceability arrangements. Pharmacy teams will often be central to ensuring that these medicines are used safely across specialist centres, local services and, where relevant, homecare pathways.

Second, real-world evidence will only be useful if data are high quality, interoperable and sufficiently complete. Small populations increase the importance of reliable baseline data, consistent outcome definitions, long-term follow-up, product traceability, adverse drug reaction reporting and data linkage across care settings. The framework should therefore align with established principles for good clinical practice, quality-by-design and real-world evidence generation.⁵⁻⁷

Third, the framework should give further consideration to comprehensive and standardised phenotyping of all potential trial participants and patients receiving treatment under the pathway. For very rare conditions, particularly where only a small number of cases exist in the UK or globally, detailed baseline and longitudinal characterisation may be essential to interpret treatment effects, support comparability across patients, and enable responsible use of external controls, natural history data and real-world evidence. This should include relevant clinical, biochemical, genomic, functional and patient-reported parameters, proportionate to the condition, treatment modality and burden on patients and families.

Fourth, the burden on patients, families and carers should be considered. Participation in long-term monitoring can involve travel, repeated assessments, uncertainty and emotional burden, particularly for children, disabled people and those with progressive or life-limiting conditions.

Fifth, reimbursement and commissioning alignment remains a major practical challenge. Regulatory flexibility alone will not deliver patient benefit unless there are credible routes to funded access, appropriate service capacity and equitable availability across the UK. The framework recognises that reimbursement sits outside MHRA's remit and that early engagement with system partners will be important.^{1,10}

Finally, non-commercial and academic developers may face different barriers from industry, including regulatory fees, trial sponsorship capacity, manufacturing support and limited incentives for repurposed off-patent medicines. These should be considered explicitly.

3. Patient engagement is a key feature of the framework. How can patients and their families and carers contribute most effectively to the regulatory process? Please explain your answer. Optional.

Patients, families and carers should contribute from the earliest stages of development and throughout the product lifecycle, not only at the point of consent. Their input is particularly important in rare diseases where clinical evidence may be limited and where benefit-risk decisions require a clear understanding of lived experience, disease burden and meaningful treatment outcomes.

Patients and carers can contribute most effectively by:

- working with developers and regulators to identify outcomes that are meaningful in daily life, including patient-reported outcomes and functional measures;
- advising on the feasibility and acceptability of proposed evidence-generation approaches including trials where feasible, registries, natural history studies, external controls and long-term follow-up and on recruitment burden and acceptable uncertainty;
- co-designing patient-facing information, consent materials and lay summaries;
- helping define acceptable benefit-risk trade-offs, especially where there is high unmet need;
- supporting registries, long-term follow-up and real-world evidence collection;
- contributing to pharmacovigilance by reporting suspected adverse drug reactions and treatment experiences.

Engagement must be representative, accessible and adequately supported. This includes involving children and young people where appropriate, carers, people with cognitive or communication needs, and communities that may be underrepresented in genomic and clinical datasets. Participants should be given adequate notice, accessible information, reimbursement for time and expenses, and feedback on how their input has influenced decisions.

The framework rightly states that informed consent should be an ongoing process, with regular opportunities to ask questions, updates at key evidence milestones, neutral lay information and the ability to revisit decisions as evidence evolves.¹

The next section has nine questions about elements of the framework

4. A fundamental question is how much evidence is enough for a decision to be made on the use of a medicine? Using novel approaches to evidence generation the pathway aims to strike a balance between the nature and amount of data needed to support the safety and efficacy of a treatment before patients can access it and the time it takes to generate that data. Do you agree that the proposed approach has found the appropriate balance?

Answer to select: Agree.

Please explain your view on this fundamental consideration. Optional.

We agree with the direction of travel. A risk-proportionate approach is necessary in rare diseases because conventional randomised controlled trials, large confirmatory studies and validated endpoints may not be feasible. The proposed Investigational Marketing Authorisation, supported by clinical trial methodology, prior knowledge, real-world evidence and structured monitoring, provides a pragmatic way to enable earlier access while evidence continues to mature.¹

However, we would not characterise the balance as fully resolved until further operational detail is available. The final framework should clarify minimum evidence expectations, including expectations for detailed baseline and longitudinal phenotyping where this is necessary to interpret outcomes in very small populations. It should also clarify the role of surrogate endpoints, standards for real-world evidence, stopping or restriction criteria, review cadence, responsibilities across the clinical trial and authorised-use phases, and how uncertainty will be communicated to patients and healthcare professionals.

The framework should make clear that flexibility does not mean lower standards. Rather, the evidential standard should be proportionate to the severity of the condition, unmet need, biological plausibility, product risk, available alternatives and feasibility of evidence generation. This is consistent with ICH principles for good clinical practice and clinical study design.^{5,6}

5. Defining rarity is a challenge due to the limitations of using a fixed prevalence (frequency) limit, changing disease classifications and the evolving nature of genetic and molecular disease definitions. Whilst retaining some flexibility, the framework is targeting diseases with a prevalence of around 1 in 50,000 of the UK population, which is deliberately distinct from the orphan designation threshold of 25 in 50,000. Do you consider that this threshold is appropriate to encourage development of treatments for rare diseases?

Answer to select: Agree.

Please provide your rationale if another threshold should be considered. Optional.

We agree that a threshold of around 1 in 50,000 is a reasonable starting point for this framework, provided flexibility is retained. The proposed threshold appropriately focuses the framework on conditions where conventional development routes are most likely to be impractical, while remaining distinct from the broader orphan designation threshold. The threshold and its operation should be reviewed periodically in light of implementation experience, evolving disease classifications, diagnostic advances and changes in the feasibility of evidence generation.

The framework should avoid being applied too rigidly. In some cases, molecular stratification, private variants, ultra-rare subgroups, severity, lack of alternatives and feasibility of trial recruitment may be more relevant than prevalence alone. Conversely, the framework should include safeguards against artificial subdivision of conditions solely to access a more flexible route.

The final guidance should clarify how prevalence should be calculated, what data sources are acceptable, how UK-wide and devolved-nation data should be considered, and how the framework will handle conditions where robust UK prevalence data are not available. Where UK-specific evidence is insufficient, applicants should be able to use robust international epidemiological or registry data, provided that the relevance and transferability to the UK population are justified. The draft framework already recognises that applicants should justify UK prevalence data and that a proportionate approach may be needed where such evidence cannot be generated, including in extremely rare conditions.¹

6. Do the Scientific Opinion and Designation steps add a tangible incentive for research and development of rare therapies? Are they best delivered at predefined points or on a flexible basis depending on the individual development? Please explain your rationale. Optional.

Yes. Scientific Opinion and Designation steps could provide a tangible incentive by giving developers, academic groups, funders and healthcare system partners earlier clarity on regulatory expectations. This is particularly valuable where development programmes are uncertain, costly or dependent on small patient populations.

A positive Scientific Opinion or Designation may also support investment decisions, trial planning, patient engagement, manufacturing planning and early dialogue with reimbursement bodies. The framework indicates that these steps can help clarify regulatory expectations, evidence-generation strategies and development plans for products using the rare disease pathway.¹

We recommend a hybrid approach: key expectations and decision points should be predefined to provide predictability, but the timing and content of Scientific Opinion and Designation steps should remain flexible depending on the product, evidence maturity, modality and patient population. This flexibility will be particularly important for platform technologies, repurposed medicines and individualised therapies.

7. Do you agree that open registration of all clinical trials which are linked to the pathway and transparent sharing of safety and efficacy data—especially from early-phase studies—will help in any of the following ways? Optional.

Answers to select:

To foster public trust;

To enhance scientific learning;

To support sustainability of the framework;

To support development of treatments for rare and more common conditions.

Please add any further comments here. Optional.

Yes. Open registration of all clinical trials linked to the pathway and transparent sharing of safety and efficacy data should be core expectations of the framework. Transparency is particularly important where early access is based on limited evidence and where each participant's data may have substantial scientific value.

Registration and reporting can help reduce duplication, minimise selective reporting, support international learning, improve patient and public trust, and enable better aggregation of evidence across very small populations. In the UK, the Health Research Authority states that registering research in a public registry is a first step in research transparency, and amended clinical trials regulations introduced new legal requirements for transparency in clinical trials of investigational medicinal products from 28 April 2026.⁸

Transparency should be implemented in a way that protects patient confidentiality, particularly in ultra-rare conditions where combinations of clinical, genomic and demographic information may make individuals identifiable. In some settings, complete anonymisation may not be achievable without substantially reducing the scientific value of the data. Any residual risk of re-identification should therefore be minimised through data minimisation, controlled access, appropriate disclosure controls and governance; assessed explicitly through research ethics and information-governance review; and explained clearly during consent. The final framework should specify proportionate transparency requirements, including accessible plain-language summaries for patients and carers.

8. Proposals for managing market exclusivity have not been addressed in the framework document. How should market exclusivity be managed to balance the potential benefits of incentivising investment with the risk of creating unintended barriers to future innovation? Optional.

Market exclusivity should be managed through transparent, proportionate and evidence-linked criteria. It should incentivise genuine innovation and evidence generation, but should not create unnecessary barriers to future research, repurposing, competition, supply resilience or patient access.

We recommend that market exclusivity should not be automatic solely because a product enters the framework. Instead, decisions should consider the level of unmet need, degree of therapeutic innovation, investment risk, quality and transparency of evidence generation, contribution to shared knowledge, commitments to long-term data collection, supply obligations and access commitments.

For individualised therapies and platform technologies, exclusivity should be carefully defined to avoid blocking other developers from using similar scientific approaches where they generate their own evidence or target different variants, populations or products. For repurposed or off-patent medicines, alternative incentives may sometimes be more appropriate than strong exclusivity, particularly where excessive exclusivity could raise costs or restrict access.

The framework should also align with the existing UK orphan medicines regime, under which orphan market exclusivity is indication-specific and subject to defined legal conditions.⁹ Where public or charitable funding has substantially contributed to development, this should be reflected in proportionality, transparency and access expectations.

9. Should decisions regarding market exclusivity and reimbursement be made on the basis of a combination of pre-determined transparent criteria rather than fixed prematurely within the framework? Optional.

Yes. Decisions regarding market exclusivity and reimbursement should be based on pre-determined, transparent criteria rather than fixed prematurely within the framework.

The criteria should be established early enough to provide predictability for developers and public confidence for patients and the NHS, but should allow case-by-case assessment of therapeutic value, evidence maturity, unmet need, affordability, supply reliability, impact on future innovation and patient access.

Reimbursement decisions should remain distinct from regulatory approval, but early alignment between MHRA, NICE, NHS England, devolved administrations, patient organisations and professional bodies is essential. The framework correctly notes that an IMA would not itself imply reimbursement or routine commissioning, which remain the responsibility of separate bodies.^{1,10}

Managed access arrangements, outcome-based agreements and structured post-authorisation evidence generation may be appropriate where there is substantial uncertainty at the point of early access. These arrangements should include clear responsibilities for data collection and medicines optimisation.

10. The scope of the framework includes authorised medicines that are being repurposed (being used to treat a different condition). Do you agree that the framework is suitable for these cases?

Answer to select: Tend to Agree.

Please tell us why you have given that response. Optional.

Repurposing authorised medicines can be an important route to improving treatment options for rare diseases, particularly where a medicine has a well-characterised safety profile from substantial use in other indications and there is a plausible biological rationale for the proposed rare-disease use.¹

However, suitability and evidence requirements should be assessed case by case and should reflect the extent, quality and relevance of prior clinical exposure. Extensive experience in other indications may reduce the need to duplicate established safety evidence. It does not remove the need to evaluate risks arising from a different dose, formulation, route, duration, target population, disease biology, organ impairment, concomitant medicines or monitoring context. Requirements should therefore be proportionate to the residual uncertainty and should focus new evidence generation on questions that are material to the proposed use.

The framework should also address practical incentives for repurposing, especially where the medicine is generic, off-patent or unlikely to attract a commercial sponsor. Academic, NHS or charity-led repurposing may require support with sponsorship, regulatory fees, evidence generation, pharmacovigilance and manufacturing or supply arrangements.

Pharmacy input will be particularly relevant for repurposed medicines, including medicines optimisation, prescribing guidance, formulation availability, supply continuity, adherence, monitoring and patient counselling.

11. Below we have listed six elements of the proposed framework. To what extent do you agree with each of these as an important element? We are interested to hear of your further thoughts on them.

11a. An adaptive regulatory pathway for very small populations

Answer to select: Very important.

Further comments. Optional.

An adaptive regulatory pathway is essential where conventional development is not feasible. Here, “adaptive pathway” should refer to a staged, lifecycle-based regulatory approach in which evidence requirements and authorisation conditions can be refined as data mature; it should not be interpreted as requiring an adaptive clinical trial design. Adaptive, Bayesian or other innovative trial designs may be valuable evidence-generation tools where they are prospectively specified, statistically robust and appropriate to the disease and available population, but they will not be suitable or feasible in every case. The pathway should enable earlier access while maintaining clear benefit-risk review points, predefined evidence requirements, patient communication and the ability to modify, restrict or withdraw access if emerging data do not support continued use.¹

11b. Defined entry criteria, such as rarity, severity, unmet need and biological plausibility

Answer to select: Very important.

Further comments. Optional.

Defined entry criteria are necessary for fairness, predictability and public trust. Criteria should include rarity, severity, unmet need, biological plausibility, feasibility of conventional development, available alternatives and capacity to generate meaningful evidence. The framework should retain flexibility but avoid inconsistent or overly broad application.

11c. Early regulatory engagement and designation

Answer to select: Very important.

Further comments. Optional.

Early engagement can reduce uncertainty, prevent duplicative work and support better evidence-generation, manufacturing, pharmacovigilance and access planning. It should be accessible to academic, NHS, charity and SME developers as well as larger companies. Where appropriate, regulatory engagement should be coordinated with early research ethics and Health Research Authority engagement, and should support alignment with NICE, NHS England and devolved system partners, while preserving the distinct responsibilities of each body.¹

11d. The use of prior knowledge, platform data and adaptive evidence generation approaches

Answer to select: Very important.

Further comments. Optional.

Prior knowledge including relevant peer-reviewed scientific publications, established scientific principles, previous clinical experience, natural history studies and justified platform or manufacturing data, together with innovative trial designs and real-world evidence, is highly relevant in rare diseases. The final framework should specify expectations for relevance, data quality, transferability, uncertainty, endpoint justification and bias mitigation. Published evidence should be critically appraised rather than accepted solely because it is in the public domain, and any reliance on internal or platform knowledge should be transparent and scientifically justified. Real-world evidence should

be planned prospectively where possible and should align with recognised evidence-quality frameworks.^{1,7}

11e. Ongoing safety monitoring with periodic review

Answer to select: Very important.

Further comments. Optional.

Ongoing safety monitoring is fundamental to the credibility of this pathway, but its intensity should be risk-proportionate and should take account of the extent and relevance of prior exposure. For a well-characterised repurposed medicine, monitoring should avoid unnecessary duplication of established safety evidence and focus on uncertainties created by the new indication, population, dose, route, formulation, duration, comorbidities or concomitant medicines. For new active substances, advanced therapies and individualised medicines, more intensive and longer-term surveillance may be required. In all cases, the Clinical Monitoring Plan should define the review cadence, adverse drug reaction reporting, product and batch traceability where relevant, patient follow-up, responsibilities across care settings and escalation processes. Pharmacy teams should be recognised as key contributors to medicines safety, pharmacovigilance and product traceability.

The draft framework states that ongoing clinical and safety monitoring are required throughout investigational use and post-IMA approval, with structured review points and clear decisions to continue, modify, progress toward full marketing authorisation or withdraw the IMA.¹

11f. The potential to progress to full marketing authorisation over time

Answer to select: Very important.

Further comments. Optional.

A clear route to full marketing authorisation is essential. It provides a long-term regulatory objective, supports developer planning and gives patients and healthcare professionals greater confidence that early access is part of a structured evidence-generation pathway. The framework should also clearly describe circumstances where progression is not appropriate and where restriction or withdrawal may be required.

12. Do you have questions or observations about any aspects of the framework including those we have not specifically asked about? Optional.

We welcome the overall direction of the framework and the recognition that rare disease therapies require proportionate, patient-centred and flexible regulatory approaches. We would encourage the final framework to address the following points.

First, the role of pharmacy teams should be made explicit. Pharmacists and pharmacy professionals are central to medicines optimisation, procurement, storage, preparation, dispensing, administration governance, monitoring, adverse drug reaction reporting, patient counselling and safe transitions between specialist and local care. This will be particularly important for advanced therapies, individualised medicines, repurposed medicines and therapies requiring long-term follow-up.

Second, the framework should clarify how information will be communicated to prescribers, pharmacists, patients and carers when a medicine is authorised under an IMA rather than a conventional marketing authorisation. Clear labelling, public assessment reports, lay summaries and professional guidance will be important to avoid misunderstanding.

Third, the final framework should include more detail on real-world evidence infrastructure, including registries, interoperability, data standards, comprehensive phenotyping, product traceability, missing data, long-term follow-up and governance for data access. This should include proportionate privacy safeguards for ultra-rare conditions, explicit recognition that complete anonymisation may not always

be possible, and clear expectations for ethics review, consent, controlled access and management of residual re-identification risk.

Fourth, the framework should clarify how the proposed UK pathway will interact with analogous international regulatory approaches and evidence expectations, including those of the European Medicines Agency, the United States Food and Drug Administration and Australia's Therapeutic Goods Administration. This will be particularly important for very rare or ultra-rare conditions where there may be only a few cases in the UK or worldwide. Greater clarity on international alignment, evidence portability, common data standards and opportunities for regulatory collaboration could help reduce duplication, strengthen evidence generation and improve the sustainability of development programmes.^{13–15}

Fifth, implementation should include workforce education. Healthcare professionals will need clear guidance on the meaning of an IMA, the evidence uncertainties, consent expectations, monitoring responsibilities and reporting requirements.

Sixth, equity should be embedded throughout the framework. Access should not depend solely on proximity to specialist centres, ability to travel, digital literacy or capacity to navigate complex systems. Devolved-nation alignment will also be important to avoid variation in access across the UK. This is consistent with the wider UK Rare Diseases Framework priority of improving coordination of care and addressing health inequalities for people living with rare diseases.^{3,4}

Finally, the framework should clarify how fees and regulatory support will apply to non-commercial developers, academic groups, charities and NHS-led repurposing projects.

The last three questions ask about your view on the impact of the framework

13. In your view, will the flexibility and adaptability of the proposed pathway provide sufficient encouragement to developers of treatments for rare diseases to bring them within a regulated pathway? Please explain your answer.

Answer to select: **Agree.**

Please add any further comments here and if you do not think this aim will be achieved please explain why.

We agree that the proposed pathway is likely to encourage more developers to bring rare disease therapies within a regulated pathway, particularly where conventional development routes are not feasible. Earlier engagement, flexible evidence requirements, modular assessment and the proposed IMA could reduce uncertainty and support more efficient development. The draft framework indicates that targeted trials, smaller datasets and earlier go/no-go decisions may reduce late-stage attrition and capital demands.¹

However, the extent of developer uptake will depend on implementation. Key factors will include clarity of eligibility, predictability of advice timelines, proportionality of fees, transparency around market exclusivity, alignment with reimbursement, support for non-commercial developers, and confidence that data generated under the framework will be acceptable internationally. For very rare conditions with only a few cases in the UK or worldwide, developers are more likely to use the pathway if there is clarity on how evidence generated through the UK framework may be recognised, reused or aligned with regulatory expectations in other jurisdictions, including the EMA, FDA and TGA.^{13–15}

The framework should also ensure that incentives apply to repurposed medicines and academic or charity-led development, not only to commercially attractive advanced therapies.

14. In your view, will the pathway achieve its aim of supporting innovation while safeguarding patients and maintaining confidence in regulatory decision-making?

Answer to select: Tend to agree.

Please add any further comments here and if you do not think this aim will be achieved please explain why. Optional.

We tend to agree. The pathway has the potential to support innovation while safeguarding patients, provided the final framework maintains robust safeguards and transparent decision-making.

Patient protection should be underpinned by independent ethics review, proportionate application of Good Clinical Practice, clear evidence requirements, transparent benefit-risk rationale, ongoing clinical monitoring, product traceability, pharmacovigilance, and defined criteria for continuation, modification, restriction or withdrawal. The draft framework appropriately states that granting an IMA enables conditional access while further evidence is generated, but does not lower regulatory standards.¹

Maintaining public confidence will also require honest communication about uncertainty. Patients, families, carers and healthcare professionals should understand what is known, what remains uncertain, what monitoring is required, and what circumstances could lead to changes in access.

15. In your view could this framework reduce barriers to patient access, including time and cost, and in turn help reduce overall treatment costs?

Answer to select: Agree.

Please add any further comments here and if you do not think this aim will be achieved please explain why.

We agree that the framework could reduce some barriers to patient access, particularly by shortening development timelines, enabling earlier regulatory engagement, reducing duplicative evidence requirements and supporting adaptive evidence generation. This may reduce some development costs and enable earlier access for patients with high unmet need.

However, reduced development time will not automatically translate into lower overall treatment costs. Many rare disease therapies, particularly advanced or individualised therapies, may remain expensive because of manufacturing complexity, small patient populations, long-term monitoring requirements and specialist service delivery needs.

To realise access and cost benefits, the framework should be aligned with reimbursement, commissioning, managed access, data collection and service readiness. It should also encourage evidence reuse, transparent pricing discussions, outcome-based arrangements where appropriate, and avoidance of unnecessary exclusivity barriers.

The framework should therefore be evaluated not only by speed of authorisation, but also by equitable patient access, safety outcomes, evidence quality, long-term value and system affordability.

Equality Impact

Do you think the proposals risk impacting people differently, or could impact adversely on any of the protected characteristics covered by the Public Sector Equality Duty set out in section 149 of the Equality Act 2010 or by section 75 of the Northern Ireland Act 1998? Optional.

Answer to select: Yes.

The proposals have the potential to improve equity by creating clearer routes to regulated access for patients with rare diseases. However, there are also risks of differential impact if implementation is not carefully managed.

Rare disease populations include children, disabled people, people with cognitive or communication needs, carers, and families facing substantial practical and financial burdens. Access could vary by geography, ability to travel to specialist centres, digital access, health literacy, language, socioeconomic status and availability of specialist services. Genomic and real-world datasets may also underrepresent some ethnic groups, which could affect evidence generation and interpretation.

We recommend that the final framework includes an explicit equality impact approach, accessible patient materials, inclusive patient engagement, support for travel and remote follow-up where appropriate, devolved-nation alignment, and monitoring of access and outcomes across relevant protected characteristics where data collection is lawful, proportionate and ethical. This would support the aims of the Public Sector Equality Duty and the equality duties applying in Northern Ireland.^{11,12}

Satisfaction Survey

The Rare Disease Framework document was easy to understand. Optional.

Answer to select: Agree.

It was easy to contribute to this consultation. Optional.

Answer to select: Agree.

References

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