Creating a fit-for-purpose evaluation process for ultra-orphan medicines

WHAT ARE RARE AND ULTRA-RARE DISEASES?

Very rare diseases, often referred to as ultra-rare diseases, affect an even smaller portion of the population, however, there is currently no formal, agreed definition of an ultra-rare disease.

A RARE DISEASE AFFECTS LESS THAN 5 in 10,000 OF THE GENERAL POPULATION.¹

NHS ENGLAND DESCRIBES TREATMENTS FOR ULTRA RARE DISEASES AS HIGHLY SPECIALISED SERVICES. THESE ARE USUALLY PROVIDED TO NO MORE THAN 500 PATIENTS A YEAR.²

Treatments for rare and ultra-rare diseases are often referred to as orphan and ultra-orphan medicines respectively. These are more common, internationally recognised terms for treatments for rare and very rare conditions.

ASSESSING ORPHAN AND ULTRA-ORPHAN MEDICINES

NICE is the national body responsible for assessing new treatments that could be made available through the NHS in England.

It is often measured in terms of the person’s ability to carry out the activities of daily life, freedom from pain, and mental disturbance.¹⁰

NICE glossary

As part of an assessment of new medicines, NICE will use a cost-effectiveness measure known as a Quality-Adjusted-Life-Year (QALY) measurement to calculate the incremental benefit of a new technology against a pre-existing standard of care. A QALY measures the state of a person’s health in which the benefits, in terms of length of life, are adjusted to reflect the quality of life.

ABOUT RARE DISEASES

75% OF ALL RARE DISEASES AFFECT CHILDREN³

35% OF DEATHS IN THE FIRST YEAR OF LIFE ARE CAUSED BY RARE DISEASES⁴

30% OF RARE DISEASE PATIENTS DIE BEFORE THE AGE OF 5⁵

4.8 YEARS IS APPROXIMATELY THE MEAN DURATION FROM SYMPTOM ONSET TO AN ACCURATE DIAGNOSIS ⁶

80% OF RARE DISEASES HAVE A GENETIC COMPONENT AND ARE OFTEN SEVERE, CHRONIC, AND LIFE-THREATENING⁶

THERE ARE BETWEEN 6,000-8,000 KNOWN RARE DISEASES⁷

UP TO 95% OF RARE DISEASES DO NOT HAVE APPROVED TREATMENTS⁸

APPROPRIATENESS OF COST-PER-QALY TO ASSESS ULTRA-ORPHAN MEDICINES

There has been widespread agreement amongst industry and the rare disease community of patients and advocates that it is inappropriate to rely solely on cost-effectiveness measures when assessing ultra-orphan medicines. This is because it is very hard to generate the necessary evidence levels required for an accurate cost-effective analysis for treatments for rare disease patients, meaning that patients can face delays in access or not receive a treatment at all.

This was recognised by NICE when, following the Health and Social Care Act of 2012, they developed a new dedicated process to evaluate ultra-orphan medicines. This is known as the Highly Specialised Technology (HST) process.

This infographic has been produced by the BioIndustry Association Rare Disease Industry Group (BIA RDIG) on behalf of the following pharmaceutical companies: Alexion, Alnylam, Pfizer, PTC Therapeutics, Sanofi, Vertex, who constitute the BIA RDIG and have commissioned and funded this work.
CREATING A FIT-FOR-PURPOSE EVALUATION PROCESS FOR ULTRA-ORPHAN MEDICINES

Following the changes to the NICE process, the Rare Disease Industry Group are calling on the Government to introduce a fit-for-purpose method for assessing medicines for patients with ultra-rare diseases. This fit-for-purpose process should:

1. Put patients’ needs at the centre and take the widest possible view of value. It should not prioritise cost-effectiveness over a holistic assessment of value.

2. Hold ministers accountable for ensuring equitable access to medicines for patients with ultra-rare conditions.

3. Identify clear and consistent criteria to decide which ultra-orphan medicines are evaluated under a HST programme.

4. Involve and develop specialist knowledge of ultra-orphan medicines and ultra-rare conditions in the evaluation process.

5. Be flexible in recognising the data limitations associated with ultra-orphan medicines.

The reforms could stop the flow of new rare disease medicines because prior to the introduction of the reforms, no medicine that successfully went through HST assessment would have met the current threshold.11

THE RARE DISEASE INDUSTRY GROUP

The BIA Rare Disease Industry Group (RDIG) represents a group of innovative bioscience companies that specialise in treatments for rare and ultra-rare diseases. The RDIG is committed to developing thinking that can pragmatically inform and support the challenge of ensuring patient access to treatments for rare and ultra-rare conditions, sometimes referred to as orphan and ultra-orphan medicines.

In particular, following changes to the NICE Highly Specialised Technologies (HST) process the RDIG wants to work with partners to achieve a fit-for-purpose evaluation process for ultra-orphan medicines to ensure patients with ultra-rare diseases have equal access to the most innovative medicines. The campaign works under the umbrella of the BioIndustry Association, the UK trade association for biotech companies.

REFERENCES


5. Ibid.


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