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# Recommendations for Gene Therapy Patient Referrals

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## Acknowledgements

These recommendations have been drawn from work on gene therapy patient referrals by the Duchenne's Muscular Dystrophy (DMD) Hub<sup>1</sup> and other relevant work on patient referrals from NHS England<sup>2</sup>, Pfizer<sup>3</sup> and Royal College of General Practitioners<sup>4</sup>.

Gene therapies have the potential to be a life-changing treatment option for patients. As the availability and demand for these therapies broadens, efficient processes for referring patients are required to ensure access and maximise opportunities for clinical sites to deliver treatment.

The processes for patients receiving gene therapy are variable. Referring a patient often relies upon local infrastructure and specialist knowledge rather than accepted, standardised best practice. The healthcare community recognises this and recommendations to improve gene therapy patient referrals have been proposed, this paper collects these recommendations to give a consolidated view for gene therapies as a whole.

The recommendations and requirements for effective gene therapy patient referrals can be categorised into four main areas: infrastructure, equity of access, coordination, and engagement.

## Recommendations

### *Infrastructure*

- ❖ Increase investment in dedicated staff, facilities, and equipment for gene therapy referrals.
- ❖ National, standardised, gene therapy referral pathways and a single NHS referral portal for each indication would be advantageous.
- ❖ National multidisciplinary panels to aid patient referrals, monitor long-term safety profiles and advise on the management of side effects.
- ❖ Develop and implement flexible and streamlined treatment models between treatment and referring sites that minimise disruption to the patients.

### *Engagement*

- ❖ A harmonised national approach to communication between treatment sites, referring sites and patients.
- ❖ Clinicians, regulators, and patient organisations to share responsibility for managing patient expectations around gene therapy referrals.

### *Equity of access*

- ❖ Gene therapy treatment site selection based on patient equity of access, disease prevalence, site geography, experience, and capacity.
- ❖ Procedures ensuring equitable access to gene therapy, regardless of patient proximity to treatment sites, should be defined in a code of practice.

### *Coordination*

- ❖ Agreed plans between the treatment and referring sites that allow for flexibility.
- ❖ Implement patient coordination strategies between treatment and referring centres that address post-treatment activities, capacity, and cost implications.

Gene therapy patient referrals should ensure appropriate communication, infrastructure and processes are in place that prioritise clinical expertise and patient equity of access. Investment and collaborative working between healthcare professionals, industry and NHS decision makers is needed to coordinate the implementation of referral pathways which enact recommendations cited here.