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Cell therapies for Duchenne muscular dystrophy: some ethical issues for personalised medicines

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Duchenne muscular dystrophy (DMD) is a chronic, complex, genetic childhood disease affecting boys, where sufferers typically die in their 20s. DMD is caused by mutations in the dystrophin gene and trials are currently underway with exon skipping, a therapy which works by patching the mutation thereby allowing production of a functional gene. The dystrophin gene contains 79 exons and mutations can be found in one or more exons. Treatment must be targeted to the specific exon which is at fault and each one therefore requires a different exon skipping chemistry. The success of trials with exon skipping will present patients and physicians with the possibility of a personalised medicine and with that, a number of ethical issues. Firstly, equity and harm by omission. Exons for the most common mutations will be tested first and researchers assert that conforming to the existing regulatory pathway for every exon, it will not be cost effective. This raises the real possibility of those children with rarer exons being denied a potentially lifesaving treatment. Secondly, if therapies are developed for the rarer mutations, risk and uncertainty become more difficult to assess. In some cases a boy may be his own control in a RCT and the boundaries between experiment and therapy become blurred. Thirdly, justice and minimum entitlement: given that minimum entitlement in this case is likely to be very expensive (the comparable myozome therapy costs $300,000 per person, per year), the distribution of limited health resources throughout a population is once again under scrutiny. This paper aims to anticipate these ethical issues with a view to helping researchers, clinicians, regulators and patient organisations to find ways to ensure that the development of these life-saving therapies progresses.

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