Sexual behaviour in young people: healthy or harmful?

Many young people charged with criminal offences relating to sexual behaviour in the UK have previously been referred to children’s services, but those entering the criminal justice system are only a small fraction affected. Using the NSPCC definition of “children engaging in sexual discussions or acts that are inappropriate for their age or stage of development” the UK National Institute for Health and Care Excellence (NICE), published their first guidelines on harmful sexual behaviour among children and young people last week.

The guidelines raise many more questions than they answer. They question the basics—the interventions available, and the assessment frameworks used—which might not be generalisable to children of different ages, backgrounds, or neurodevelopmental stages, or girls. This matters because young people who do not access the right care continue to pose a risk to themselves and to others, and those wrongly assessed might have their futures indelibly marked.

Patient need versus evidence: a balancing act

On Sept 19, the US Food and Drug Administration (FDA) granted accelerated approval for eteplirsen, a new drug for the treatment of Duchenne muscular dystrophy. The decision goes against the recommendation of the FDA’s advisory committee—which earlier voted not to approve the drug, citing concerns about the quality of the evidence—and follows months of internal wrangling among FDA officials. The approval was applauded by parents and advocacy groups, who had been vigorously lobbying the FDA, but led to accusations from drug policy experts that the agency was setting a dangerous precedent by approving a drug on such limited evidence and ignoring the advice of its expert panel.

Eteplirsen restores the readability of a damaged part of the gene encoding dystrophin—absence of which causes muscular dystrophy—resulting in formation of a truncated but functional form of dystrophin. Approval was largely based on the surrogate endpoint of increased dystrophin expression in muscle biopsies from just 12 boys after 48 weeks of eteplirsen treatment. However, increases in dystrophin were modest and whether such gains are sufficient to slow functional decline remains to be seen. Sarepta Therapeutics, the drug’s manufacturer, must now undertake a 2-year randomised controlled trial to assess the clinical benefit of eteplirsen. If efficacy is not confirmed, the FDA could withdraw approval.

With few treatment options available for muscular dystrophy, parents are understandably desperate. The FDA clearly states that the functional effects of eteplirsen are not proven. Balancing patients’ needs and expectations against the weak evidence base is a difficult task. But raising hope, perhaps unrealistically, by approving drugs on such uncertain evidence is not the answer, and could even be counterproductive by jeopardising the ability to undertake placebo-controlled trials. Well designed and funded studies of the functional efficacy and safety of eteplirsen should be the way forward, and a recent Policy View highlighted the power of a collaborative effort between patients, scientists, and regulators to help develop drugs for muscular dystrophy. Patients with muscular dystrophy deserve an effective treatment—only time will tell whether the FDA’s decision was the correct one.