RESEARCH NEWS

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Another clinical trial for DMD started

Dutch biopharmaceutical company Prosensa has announced the initiation of another exon skipping clinical trial for Duchenne muscular dystrophy (DMD). The potential drug being tested is called 'PRO053' and is a 'molecular patch' designed skip a portion of the dystrophin gene called 'exon 53'. It has the potential to treat around eight percent of boys with DMD. The first patient in the trial has now had his first injection of PRO053.

This phase 1/2 clinical trial is designed to assess the safety, effectiveness and tolerability of multiple doses of PRO053 over 48 weeks. They will also study how PRO053 moves through the body for example the rate at which it is absorbed and excreted – this is called the 'pharmacokinetics'. The main measure of the effectiveness of the drug will be how far the boys can walk in six minutes before and after treatment. The initial part of the trial will be conducted at several clinical trial sites in Europe and then may be extended to additional countries in and outside of Europe.

Exon skipping is a personalised medicine - the molecular patch needs to be tailored to the specific mutation in the dystrophin gene. Prosensa now has four molecular patches in clinical trial and two more undergoing testing in the laboratory (pre-clinical testing). Rival company Sarepta Therapeutics has one molecular patch in clinical trial and three in pre-clinical testing. The chemical compositions of the molecular patches developed by the two companies are slightly different but the principal of how they work is the same.

The first molecular patches in clinical trial are those that have the potential to treat the highest number of boys with DMD. If this proves to be successful more exon skipping drugs will be made to target other regions of the dystrophin gene. In total it is thought that approximately 83 percent of boys with DMD may be able to be treated by exon skipping but this will require the development of more than 100 different molecular patches which could take some time.

Summary of exon skipping drugs being developed

Exon skipped	Proportion of boys potentially treated	Prosensa	Sarepta
44	6.2%	Phase 1/2 trial	
45	8.1%	Phase 1/2 trial	Pre-clinical
50	4.0%		Pre-clinical
51	13%	Phase 3 trial*	Phase 2b trial**
52	4.1%	Pre-clinical	
53	7.7%	**Phase 1/2 trial**	Pre-clinical
55	2.0%	Pre-clinical	

Note: The exon 51 drugs have been given the names * Drisapersen and **Etiplersen. Prosensa is collaborating with GlaxoSmithKline to conduct the Drisapersen clinical trial

Further information

- Read the Duchenne muscular dystrophy factsheet for background information on the cause of DMD and a summary of research currently underway
- Find out about the latest exon skipping clinical trial results
- Clinical trials: Your questions answered
- What is exon skipping and how does it work?
- Read Prosensa's press release

If you have any questions please contact us:

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