Muscular Dystrophy UK
Fighting muscle-wasting conditions

What needs to happen next?

The crucial next step is to ensure that Sarepta submits what is known as a Marketing Authorisation Application (MAA) to the EMA. In a recent webcast, the company has said that they plan to apply by the end of 2016. MDUK and fellow charities will be engaging with Sarepta in the coming weeks and months, and urging a broad licence application to include both ambulant and non-ambulant patients.

Once the company has applied:

- The EMA will consider approval for the drug. The group within the EMA responsible for these considerations is the Committee for Medicinal Products for Human Use (CHMP). As part of this process, patients and families will be invited to share their experiences of living with Duchenne, and the importance of access to treatments such as Exondys 51 designed to slow down the progression of the condition.
- Sarepta might be asked for further details, for example for clarifications on the clinical trial data
- Once all the information required has been gathered, a CHMP 'opinion' will be issued which will determine whether the EMA is giving approval and granting a licence for Exondys 51.
- If the EMA approves the drug, it will be up to authorities in the UK whether to make it available in hospitals here.
- In England, NICE is responsible for this decision. NICE will consider drugs either by a Health Technology Assessment or through its Highly Specialist Technologies programme (HST). Given that Duchenne drug Translarna was assessed via HST, we would push for a drug like Exondys 51 to be assessed in the same way.
- In Scotland, it is the Scottish Medicines Consortium who takes decisions on new drugs. In Wales and Northern Ireland, a separate process is used but this often follows the decision made by NICE. The Isle of Man has a separate process.

It is hard to give a definitive time frame for this whole process. For Translarna, the process took over three years. However, this was partly due to a request to the EMA for a reconsideration (they initially rejected a licence for the drug).This was followed by significant delays at NHS England, and a lengthy appraisal by NICE. MDUK has been working with NICE and NHS England – and we will be pushing to ensure that future decisions are taken with greater speed.

It will also be important to look at other ways of making the drug available more quickly – for example through the Early Access to Medicines Scheme.

Watch this **video** for more information on how the EMA interacts with patients – which has been shared by Alex Johnson at Joining Jack.