The Patient Perspective: Adaptive Licensing

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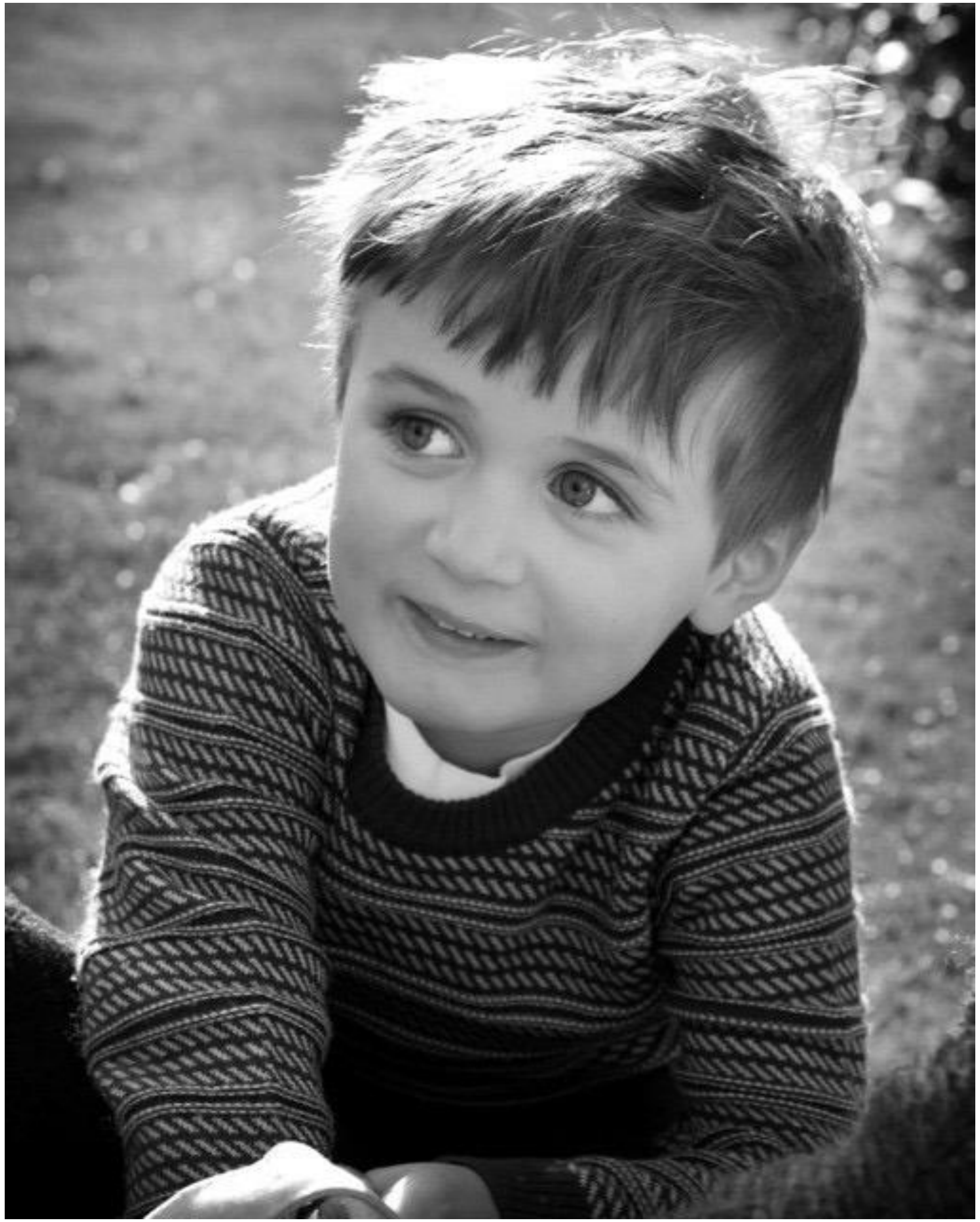
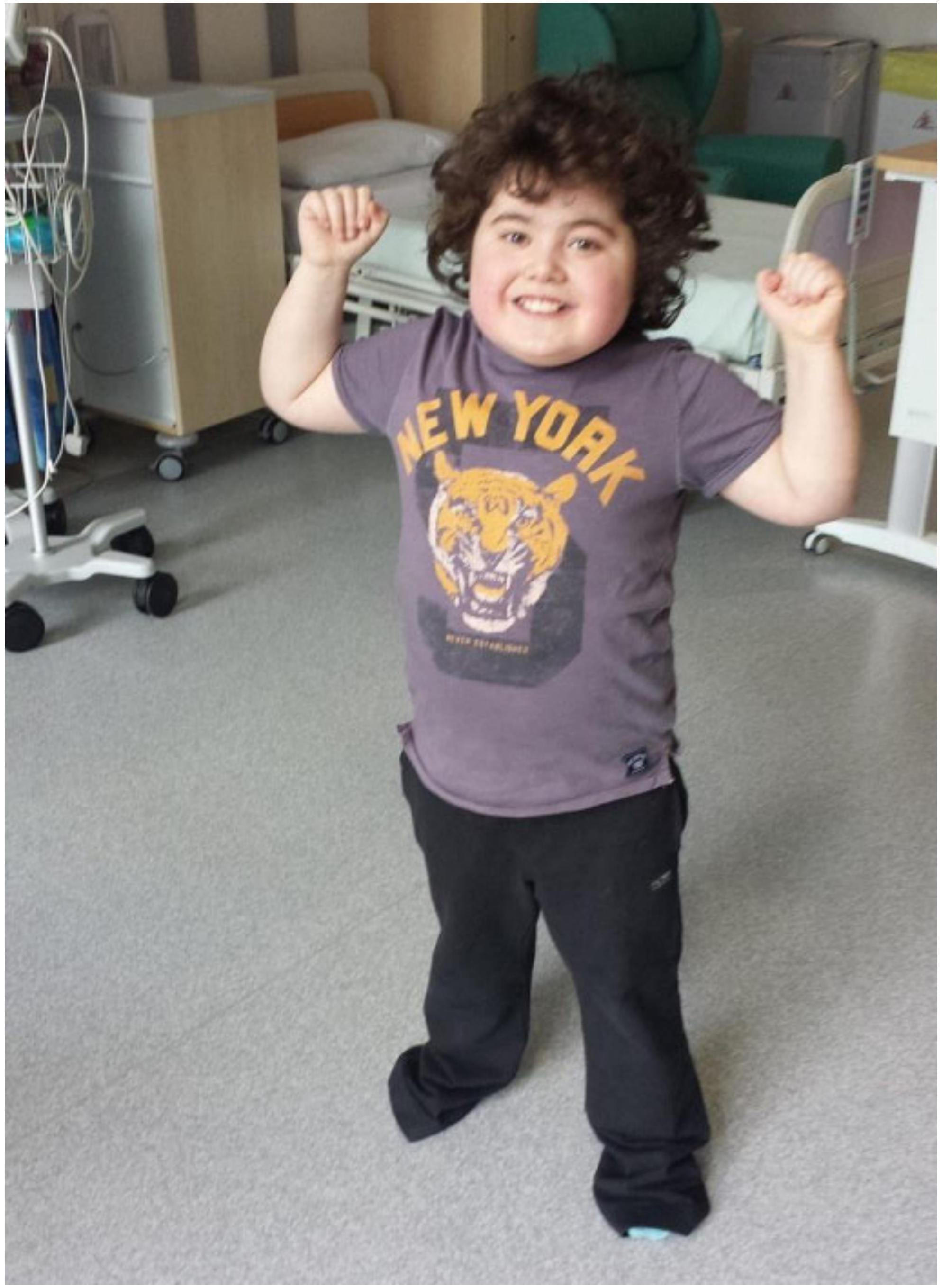
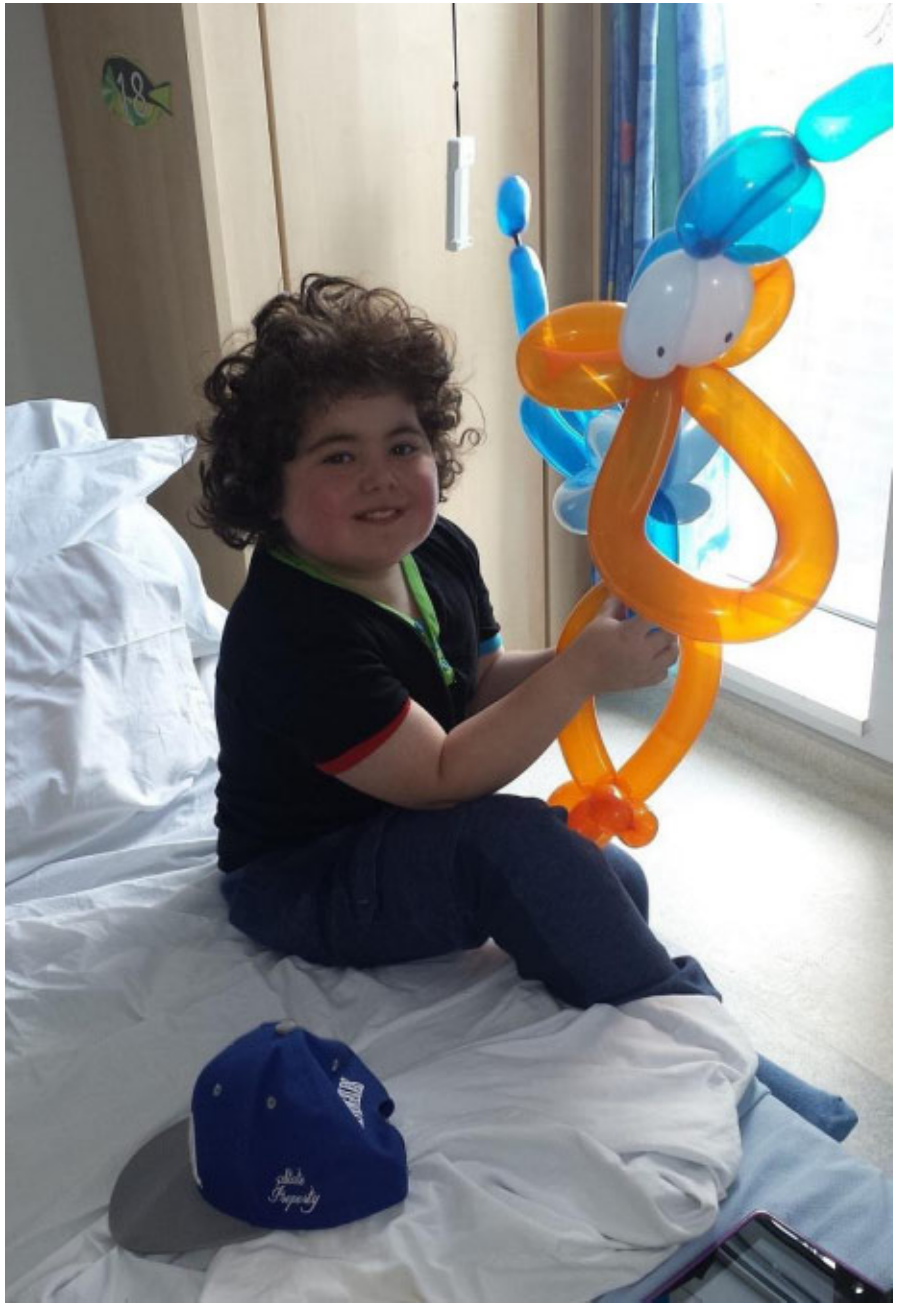
DUCHENNE
CHILDREN'S TRUST



Eli



Joey



‘Time is our enemy. We’ve taken the “risk” of putting Joey on a trial. And we would certainly take the so-called “risk” of putting him on an experimental drug that had been approved through the adaptive pathways. Because for us, the biggest risk of all, is to do nothing ’

– Tony, Joey’s dad

We are hopeful !

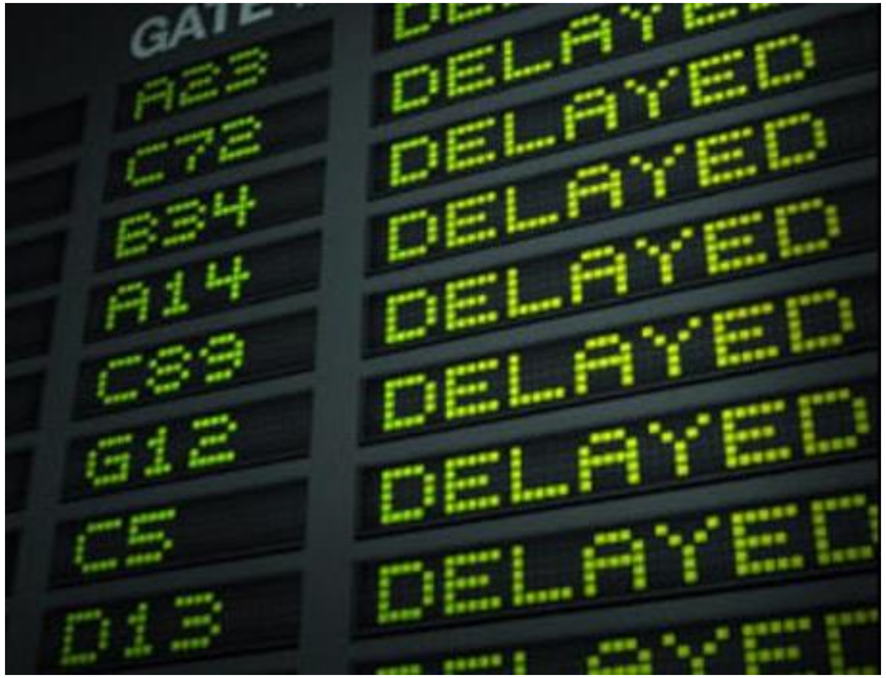
Jack

'To see drugs being trialed that are making a real difference to boys with Duchenne, and being told that I might have to wait 5 or 10 years … It's like we're on the Titanic. We can see the lifeboats in the distance – lifeboats that might get us to safety. But we're powerless.

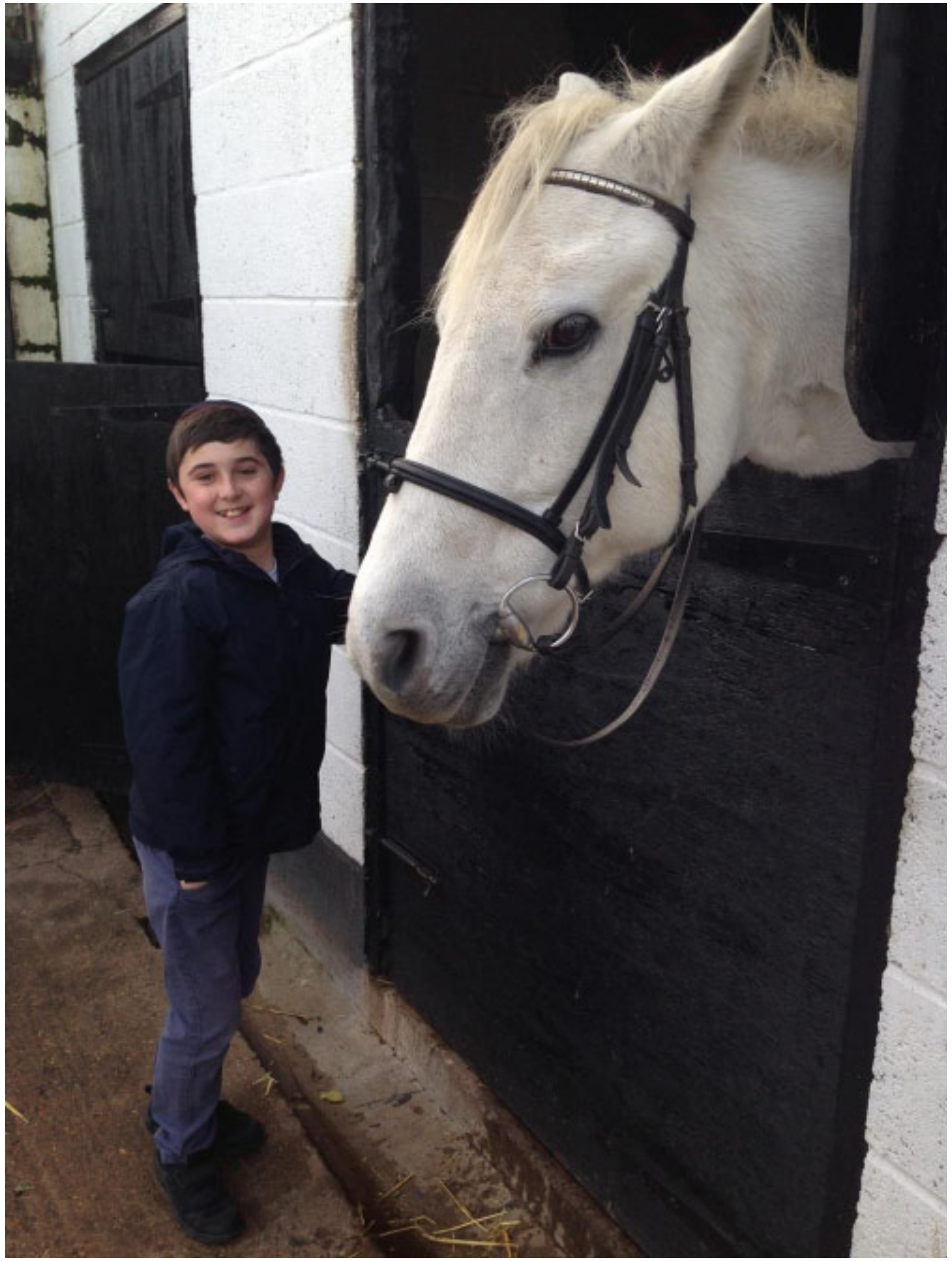
We know they can't reach us. '

– Alex, Jack's mum

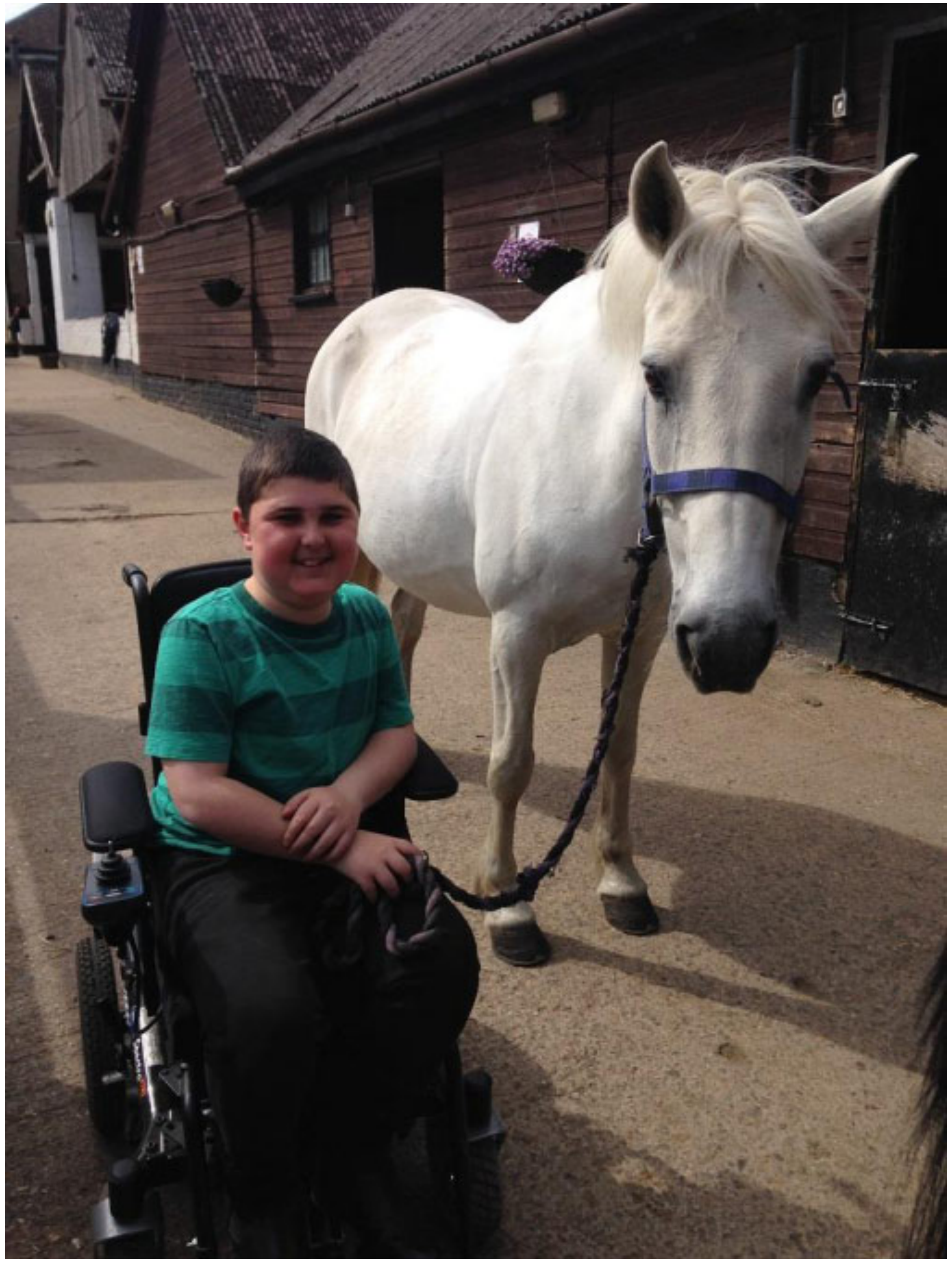
Conditional Approval –Yet Still Delays



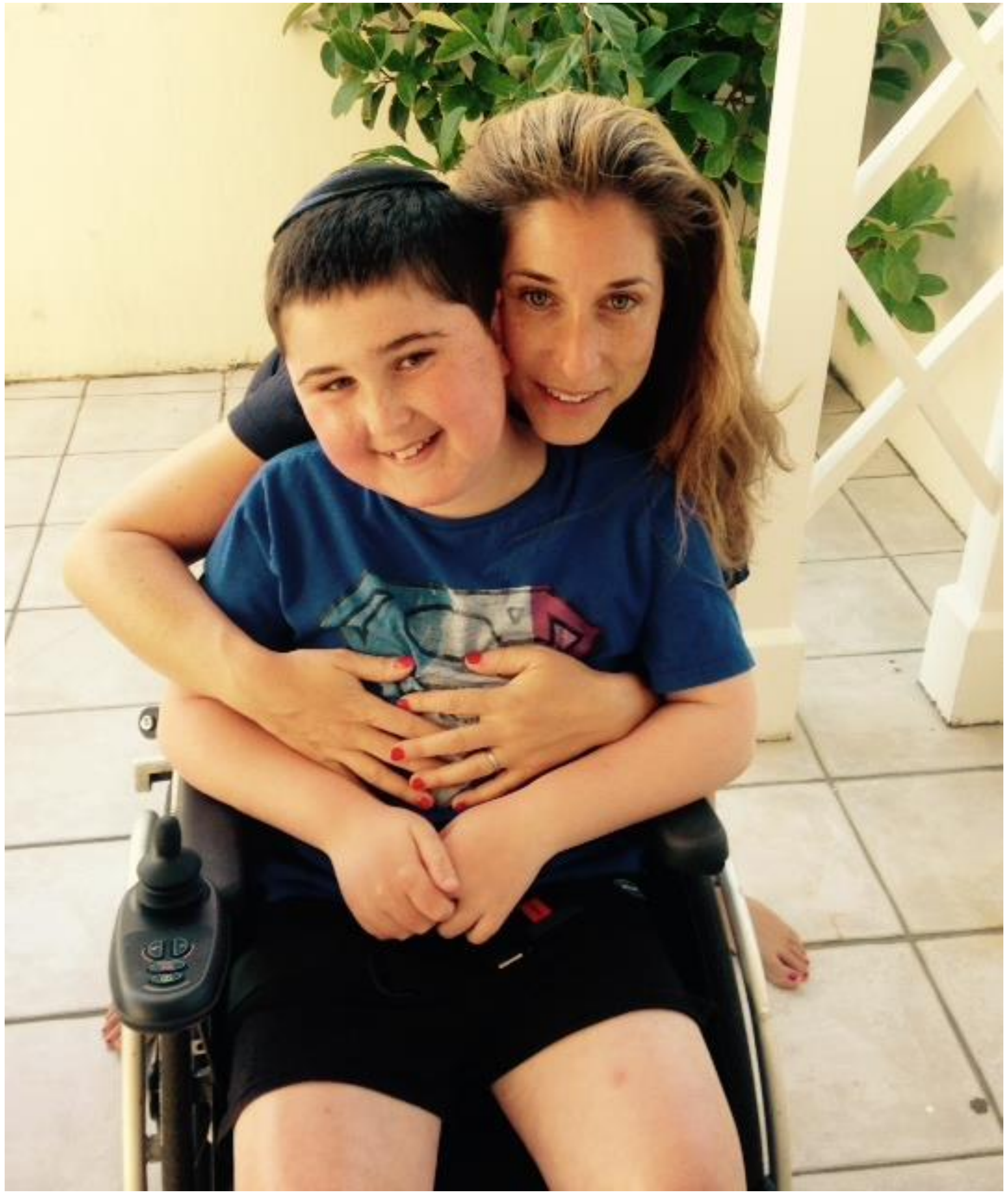
- May 2014: Ataluren given Conditional Approval
- October 2015: Reimbursement decision still not made



Gavriel, aged 11
October 2013



Gavriel, aged 12
May 2014



‘My son is 13. He understands this disease and what it will do. His views need to be listened to. Patients and carers living with Duchenne – are living with ticking clocks on their shoulders. Every day without a treatment, is a day more of muscle loss’

– Kerry, Gavriel’s mum

Patient Groups welcome the Adaptive Licensing Pilot and hope it will light and lead the way forward