

Our Zolgensma Journey: Five Years Later

Five years ago, in August 2021, my son, Ollie, was one of the first children in England to receive Zolgensma – a one-off gene therapy infusion replacing the missing SMN1 gene that SMA has taken away from him. He was only 9-months old, and had been diagnosed with SMA Type 1 when he was 15-weeks old. He had started on Spinraza and received the first four loading doses before we switched treatment paths to Zolgensma.

It was a worrying time for our family, as the drug was in its infancy and the long term side effects were still unknown. There were many preparation appointments where we needed to take Ollie up to London (a 2-hour trip one way). The date of when Ollie would receive the treatment was up in the air, so we had to put our lives on hold and keep him shielded just in case it was short notice.

The COVID-19 pandemic was still very much at the forefront of people's minds, and hospital appointments were limited to one parent/carer, as well as being careful in day-to-day life with who we came into contact with. And Ollie had just come out of a 7-week intensive care stint after catching a common cold, which put us on edge about his health stability.

Yet, we knew Zolgensma was the right treatment choice for Ollie, and we had followed its progress to becoming available on the NHS right from diagnosis. It was actually the same day that Ollie came home from the Evelina Children's Hospital in March 2021, with all his new medical equipment in tow (Bi-Pap ventilator, Cough Assist machine, Suction machine, feeding tube, etc), when news broke that the NHS had approved Zolgensma, the first gene therapy treatment, for use in children under two years of age. It then became a race against time to get him treated, as time is motor neurons, and he had already lost a lot of muscle movement.

However, this isn't just Ollie's story, as he is now one of many children who has received Zolgensma in the UK. We know that if children are treated from birth, then the onset of symptoms can be stopped in its tracks, and that's why advocating for newborn screening has been such an important part of our lives since we learnt about SMA. The same goes for access to treatments, as everyone affected by SMA deserves the right to live. So we will continue to fight with the SMA community to get the care our family and others need.



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