Hooriya's story

Rehna's daughter, Hooriya was born six weeks premature. Initially, doctors believed that Hooriya wouldn't survive, as her chest was weak and an x-ray revealed that her ribs were thin. The Royal Manchester Children's Hospital diagnosed Hooriya with the rare and sometimes fatal genetic disease, Hypophosphatasia (HPP).

My husband, Ehsan, and I were really worried when Hooriya was born early. She's our first child and was really ill. As well as not being able to breathe by herself, Hooriya was 'floppy' and less active than other babies.

The Royal Manchester Children's Hospital recognised Hooriya's condition as HPP. It's rare and I'd never heard it before. It was a really difficult time for us, and we were told that she might not make it.

But, a doctor at the children's hospital gave us hope. He explained that there was a new treatment, asfotase alfa, which had shown positive results in early clinical research studies. At the time the treatment was only available as part of research study abroad. Thankfully Hooriya was given the treatment on compassionate grounds.

We would have lost Hooriya without this new treatment.

Once Hooriya started on the medication, she started moving her arms and legs. Then, as she grew her chest got better. Hooriya was taken off the ventilator and now only needs oxygen when she goes to sleep.

Two years later, Hooriya was ready to come home. At first I worried that she might have trouble fitting into life with family and friends, but now Hooriya is a bright and happy little girl, who enjoys going to the Rainbow Centre where she can play with other children. We've come a long way. Hooriya is learning to walk and to sign (the breathing tube makes it difficult for her to talk).

Hooriya wouldn't be here if she hadn't have had the treatment. It's amazing that a small bottle of liquid - an injection that I give to Hooriya three times a week at home can make such a difference.

Learn more about research at The Royal Manchester Children's Hospital and how to get involved in research.

About Hypophosphatasia (HPP)

HPP is a rare and sometimes fatal metabolic bone disease caused by gene mutations. As a result, the body is unable to generate enough an enzyme called TNSALP (Tissue non-specific alkaline phosphatase), which is required to harden bones.

Early studies indicate that Asfotase alfa, a treatment designed to replace the missing enzyme, is well tolerated in infants and children with HPP. The Royal Manchester Children's Hospital, in collaboration with the National Institute for Health Research / Wellcome Trust Manchester Children's Clinical Research Facility, is now delivering a study to evaluate the long-term safety and effects of subcutaneous (under the skin) injections of the treatment.