

# Gene Therapy Center

Innovating care.  
Transforming lives.





# Why Choose Sidra Medicine?

Sidra Medicine's state-of-the-art facilities offer more than just world-class medical care. We reignite hope and healing for women and children around the world seeking specialized treatment. Our renowned physicians are leaders in their fields, delivering compassionate care and leading groundbreaking research that translates to better outcomes for our patients. When you choose Sidra Medicine, you are choosing an institution that is dedicated to your well-being and peace of mind throughout your healthcare journey.

## Gene Therapy Center

The Division of Genetic and Genomic Medicine at Sidra Medicine provides comprehensive genetic services including clinical evaluation, diagnosis and risk assessment; genetic counselling; and the advanced management for a variety of genetic disorders for pediatric patients. The division also offers ongoing multidisciplinary care for patients with genetic conditions and helps them access resources and treatment programs personalized to their condition.

## Pioneering Gene Therapy Treatments

Sidra Medicine is a regional leader in gene therapy treatments with an extensive program and an experienced team that reflects our commitment to providing innovative, safe, and effective treatments for genetic and rare diseases.

The Gene Therapy Clinic under the Division of Genetic and Genomic Medicine at Sidra Medicine is dedicated to offering life-saving treatments for rare genetic disorders and serves as a beacon of hope in the region. Our multidisciplinary team, including geneticists, neurologists, therapists, pharmacists, and other subspecialists, is committed to delivering comprehensive care and life-changing therapies.

## Duchenne Muscular Dystrophy (DMD) Care

Elevidys, developed by Sarepta and Roche Pharmaceuticals, is the first and only approved gene therapy for DMD. The treatment introduces a functional dystrophin gene to muscle and heart cells, slowing disease progression and improving muscle function. Sidra Medicine has become a key player in DMD care in the region and is the first hospital outside the United States to administer Elevidys to children over the age of six.

## Spinal Muscular Atrophy (SMA)

SMA is treatable with Zolgensma, a cutting-edge treatment developed by Novartis that halts disease progression and improves outcomes. Sidra Medicine was selected as the lead treatment center for the MENA region by Novartis and has successfully administered Zolgensma to several children.





## A Pioneer in Genetic Care

Dr. Tawfeg Ben-Omran is a renowned expert in clinical and metabolic genetics, serving as the Division Chief of Genetic and Genomic Medicine at Sidra Medicine.

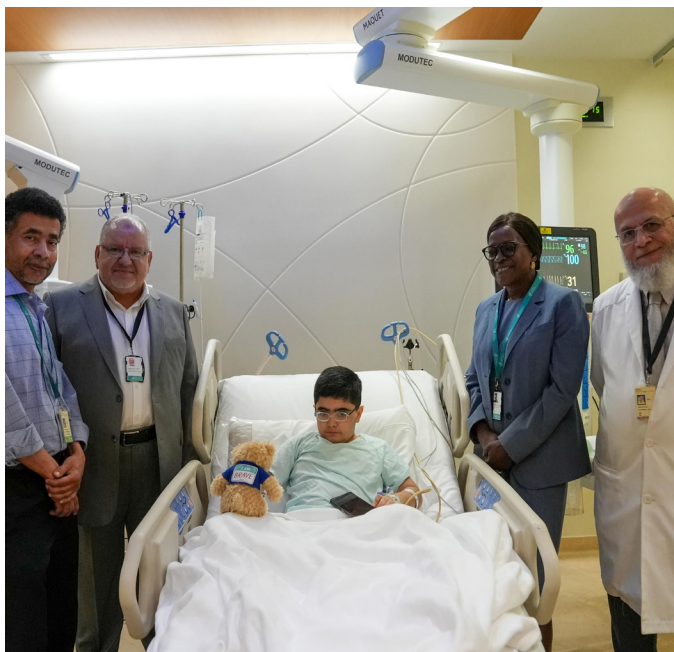
With over 100 publications, he leads cutting-edge research on enzyme replacement therapies, genetic brain malformations, and rare disorders.

A senior consultant and Professor at Weill Cornell Medicine-Qatar, he is also a distinguished visiting scientist at Boston Children's Hospital. Recognized internationally, Dr. Ben-Omran is a leader in advancing genetic care for the Arab population and holds multiple prestigious awards for his groundbreaking work.



## Changing Lives, One Child at a Time Overcoming Limits in DMD Care

Fahad, a 10-year-old boy with Duchenne Muscular Dystrophy (DMD), was part of the first group of older children to receive Elevidys gene therapy at Sidra Medicine. In late 2024, the age limit for Elevidys treatment was lifted, making Sidra Medicine the first hospital outside the U.S. to offer this therapy to children over six. Fahad's mother, initially devastated by the belief that there was no cure due to her son's age, expressed immense relief when the treatment became available. She shared, "We are grateful for the incredible care provided by the entire team at Sidra Medicine... I would like to extend special thanks to Dr. Tawfeg, Dr. Fawzi, and Dr. Khalid for their exceptional support."



## A Story of Healing, Hope, and Community Support

Malkha, a baby diagnosed with Spinal Muscular Atrophy (SMA), received life-saving gene therapy at Sidra Medicine, making it the leading hospital in the region to administer this treatment. The therapy was made possible through the collaborative efforts of Qatar Charity and the community. Malkha's parents, deeply moved by the support, expressed their gratitude, saying, "Words cannot express the generosity and kindness of the people in Qatar, Qatar Charity, and the wonderful medical team at Sidra Medicine. Alhamdulillah, that we were able to have this life-saving treatment for our baby. Thank you for giving Malkha a chance."







### **Global Patient Services**

Each year, Sidra Medicine's Global Patient Services team works with families from outside Qatar who travel to receive specialized care at the hospital. The diversity of our international patients at Sidra Medicine is a testament to our expertise. To date, the hospital has cared for patients from around the world, including Kuwait, Afghanistan, Iran, Iraq, Oman, Egypt, Turkey, Mozambique, Uganda, Kenya, South America, the United Kingdom and more. We continue to be a trusted destination for those seeking the latest in pediatric cardiovascular care, neurosurgery, neurology, urology and gene therapy treatments including SMA and DMD.

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