What do you do when your child's life is dependent on a Rs 16 crore drug?

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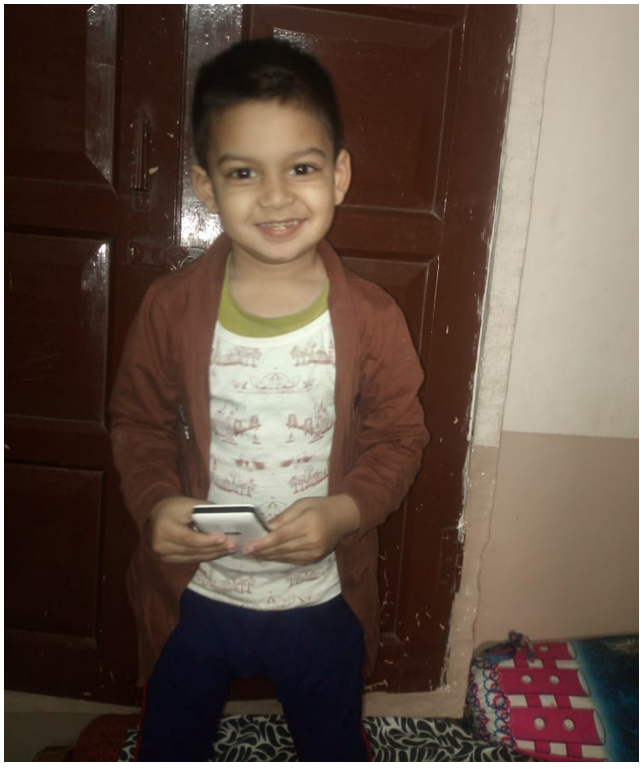
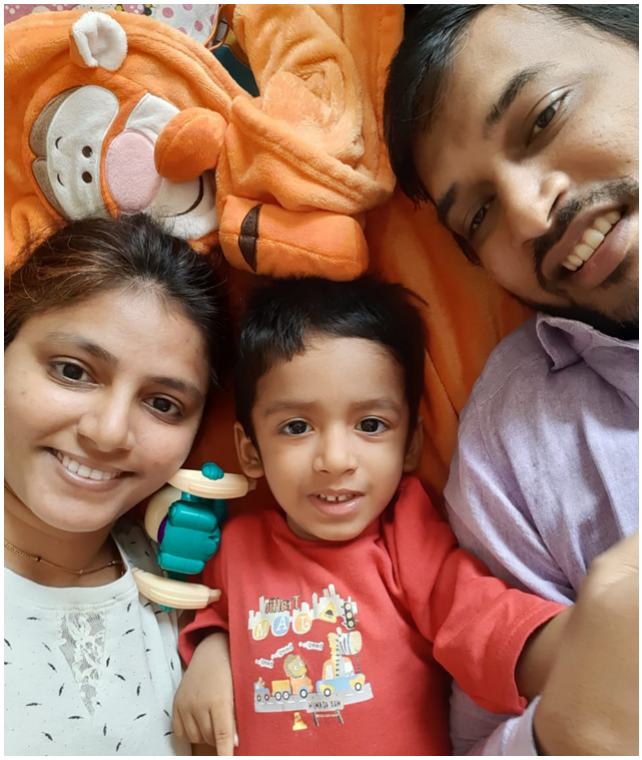


Four-year-old Mohammed Zaheer has Gaucher's disease, a rare genetic disorder. The treatment costs Rs 1 crore annually

Ayaansh was about eight months old when his family realised something was wrong — he could not crawl or sit up, struggled to have milk or food, and had no neck control. The paediatrician would say that it could be a milestone delay. "But, when he did not achieve any milestone at nine months, we visited a neurologist and were shocked to hear that he had Spinal Muscular Atrophy (SMA)," says Yogesh Gupta, Ayaansh's father.

This was in June 2019. Two years on, the family's hopes are pinned on a wonder drug - Zolgensma. However, the medicine costs — $2.125m (around Rs 16 crore) — are prohibitive. So far, the family has been able to raise Rs 1.4 crore and are requesting NGOs, philanthropists and the government to help.



Ayaansh with his parents who are trying to raise Rs 16 crore for his treatment

For Kota-based vegetable vendor and kirana store owner, Mohamad Rafiq, life delivered a nasty punch not once but twice. His first born, a girl by the name of Parveen Nisha, was born with a rare genetic disease called Gaucher's disease. In this inherited metabolic disorder, the patient is born without the enzyme which is responsible for breakdown of fat. In its absence, fat starts accumulating in the liver and spleen, making them grow in size. The child starts to look like a pregnant woman.

Parveen could be diagnosed only by the time she turned one-and-a-half and she didn't survive beyond her 5th birthday because her poor parents couldn't afford the treatment that costs about Rs 1 crore per year and is life-long. Rafiq's second born, a son, suffers from the same disease. Now 4, Rafiq's son benefited from free treatment provided by a pharma company for about six months. But his future is uncertain. While the Jaipur high court has ordered a local hospital to foot the bill for his treatment, the hospital has so far not been able to carry out the order, said Rafiq.

"After spending about Rs 30 lakh on my daughter's treatment now I have no money left to take care of my son," Rafiq said.

Both children are among the estimated 70 million Indians, who suffer from one of the 7000+ rare diseases worldwide. These are life threatening or chronically debilitating conditions that affect a very small number of people. About 80% of all these diseases are genetic in nature. These rare diseases particularly impact children, causing 35% of deaths before age 1, 10% between the ages of 1-5 and 12% between 5-15 years. According to the Indian Council of Medical Research (ICMR), a disease or disorder is defined as rare in India if it affects fewer than 1 in 2,500 people.

Parents whose children suffer from rare diseases had pinned their hopes on the long pending National Policy on Rare Diseases, which will most likely be notified by the Union Ministry of Health & Family Welfare by March 31. The policy proposes financial support of up to Rs 15 lakh per person under an umbrella scheme of the Rashtriya Arogya Nidhi

for those rare diseases that require a low-cost one-time treatment of stem cell transplant or organ transplant.

What is a Rare Disease?

It's a health condition of low prevalence that affects a small number of people compared with other prevalent diseases in the general population. In India, a disease or disorder is defined as rare when it affects fewer than 1 in 2,500 individuals.

WHO defines a rare disease as often debilitating lifelong disease or disorder condition with a prevalence of 1 or less, per 1,000 population.

For diseases that need lifelong treatment but the cost is relatively low, state governments can offer support. But for diseases whose treatment is both lifelong and high-cost, there is no cover as of now — these include Gaucher's disease, Hurler syndrome (in which the body cannot digest sugar), Hunter syndrome (in which sugar molecules build up in body tissues), Pompe disease (which damages muscle and nerve cells), spinal muscular atrophy (in which babies can't use their muscles), among others.

Patients and patient advocacy groups are unhappy with the way the policy has excluded diseases that need lifelong treatment and are expensive to treat – in the policy document classified as category III illnesses. On an average, the cost of treating these diseases can go up to Rs 1 crore per patient, per year, or as in the case of Ayaansh, can cost as much as Rs 16 crore.

Rare Diseases — What we know

- There are more than 7000+ rare diseases (RD) known/reported worldwide

- Over 350 million people worldwide are affected — around 30 million in USA, 30 million in EU, 70 million in India

- 1 in 20 Indians is affected by one of these RDs

- About 80% of RDs are genetic in origin

- 50% of RDs are onset at birth and the rest are late onset

- RDs include inherited cancers, autoimmune disorders, congenital malformations, and infectious diseases amongst others include

- Hemangiomas, Hirschsprung disease, Gaucher disease, cystic fibrosis, muscular dystrophies and Lysosomal Storage Disorders

- Majority of the diseases have no treatment and when they exist, are mostly unaffordable

- Early diagnosis is a critical challenge in RD management (Avg time 5-7 yrs)

- So far, only about 450 rare diseases have been recorded in India

Source: Organisation of Rare Diseases India

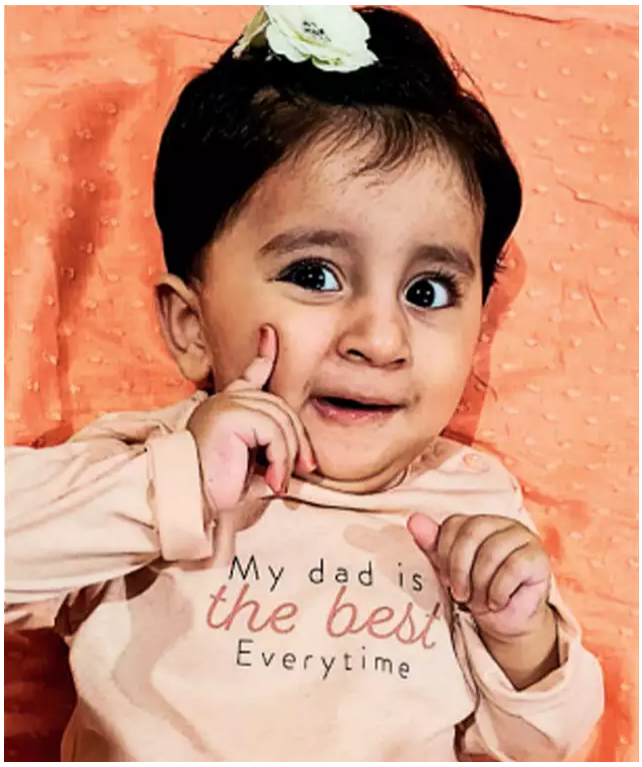
In the policy document, the government admits “keeping in view the resource constraint and competing health priorities, it will be difficult for the Government to fully finance treatment of high-cost rare diseases.” It instead suggests “creating a digital platform for bringing together notified hospitals where such patients are receiving treatment or come for treatment, on the one hand, and prospective individual or corporate donors willing to support treatment of such patients.” This has been especially suggested for category III diseases.

Prasanna Shirol, founder and president of Organisation of Rare Diseases India and whose daughter suffers from Pompe disease asked what the government’s role would be if it expects people to raise money for rare diseases. “Who will donate, who will monitor it? Treatment of rare diseases is expensive because it’s life-long. Even the wealthy can’t afford it,” said Shirol.

A 28-year-old with Gaucher’s disease who didn’t want to be named said, “It’s a cruel joke for our community. Such steep costs cannot be addressed by crowdfunding. I don’t think it’s about the money but about the government's priorities.” He is on a humanitarian programme of the pharma company that makes medicine for Gaucher’s and he will get free medicine all his life.

“The government needs to reallocate the budget. If it can keep Rs 35,000 crore for Covid, it can surely allocate Rs 500 crore for rare diseases. A cap of Rs 15 lakh makes things difficult for some patients who require more expensive treatments. A more workable solution can be to have partnership between government and charitable programmes that subsidise treatment,” said Dr Ashok Gupta, in-charge Rare Diseases Centre at J K Lon Hospital, Jaipur. He’s also chairperson of the technical committee on rare diseases, Government of Rajasthan. Promoting and developing local production of medicines needed for rare diseases can also be a sustainable option. “Importing medicines from outside makes them expensive. The government should enable local manufacturing capability for these drugs to bring down their prices,” said Dr Muhammed Asheel, executive director, Kerala Social Security Mission under Government of Kerala.

This baby can move legs after dose of Rs 16cr drug



On January 21, 14-month-old Fathima, who suffers from Spinal Muscular Atrophy (SMA), a genetic disease, got a new lease of life after receiving the drug, Zolgensma, at Bangalore Baptist Hospital. Fathima was the lucky winner of a lottery through a compassionate access programme by Novartis, the drug manufacturer.

Gradually improving, baby Fathima has been able to move her legs since she was administered 40 ml of the drug through IV fluids.

“We were extremely lucky to get the drug through the lottery from the manufacturer. Even if I had earned Rs 16 crore, I don’t know if my daughter would have lived till then,” said Mohammed Basil, her father who works as an auditor in Dubai and hails from Bhatkal, Karnataka.

“The doctors informed us about Zolgensma and the drug manufacturer’s lottery scheme. My daughter’s blood sample was sent to a lab in the Netherlands for a test before participation in the scheme,” recalled Basil.

Two months ago, Basil got a call from the hospital and was told Fathima had won the lottery. “It was nothing short of a miracle. She is very lucky to get the drug when several other patients with SMA are waiting for the same,” he said.

There were hiccups at the customs end; officials did not release the drug and it required the intervention of the doctor treating Fathima and Basil was asked to pay close to Rs 1.5 crore as integrated GST. Says Basil: “There is a need to create awareness among customs officials about the importance of such essential life-saving medicines. I request the Indian government to approve the drug in India and provide it to needy children at a subsidised price,” he told TOI.