

## Unconventional drug ‘lottery’ will hand out 50 doses of Zolgensma, world’s most expensive gene therapy

The lottery that began [recently] was not about money, or about choosing a school, or about obtaining a visa. It was about a child’s life. In this case, the children selected would receive a drug that otherwise was not available.

...

The treatment, a gene therapy called [Zolgensma](#), is designed for children like Wynter who have a neuromuscular disease called spinal muscular atrophy, or SMA.

...

[The lottery](#) was devised by the drug’s manufacturer, Novartis, to give families in those places a chance to get it through a novel form of compassionate use — a way to get medications that have not been approved — while they wait. Fifty doses are slotted to be given away [for free](#) in the first half of the year, with up to 100 total.

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Ethicists and advocates have debated the merits and the design of the unusual arrangement. Parents said that it was uncomfortable to cast their child’s fate into what felt like a sweepstakes — a kind of bizarre Willy Wonka contest in which, as Maura Blair, a Canadian mother of a child with SMA put it, “we’re talking about lives.” ...

Zolgensma costs \$2.1 million in the United States — the world’s most expensive drug.

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