

Novartis eyes give-away of costly therapy for babies

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Swiss pharmaceutical giant Novartis draw praise and criticism over its give-away

Swiss pharmaceutical company Novartis is preparing to give away 100 doses of the world's most expensive drug, which treats a rare childhood disorder, but its recipient selection process has drawn criticism.

The company announced this week that starting next month, its AveXis unit will begin distributing doses of Zolgensma, a one-time gene treatment for spinal muscular atrophy, also known as SMA.

The disease affects about 1 in 10,000 births, and which results in death or the need for permanent ventilation by the age of two in 90 percent of cases.

Novartis said a total of 100 doses—which cost around \$2 million a pop—would go to eligible patients who are "under the age of two and are a citizen or legal resident of a country where the therapy is not yet approved by regulatory authorities."

The therapy was approved by US regulators in May, but approval in Europe and Japan for instance has been delayed until next year.

Novartis stressed in a statement that AveXis so far had only one facility licensed to produce the therapy, and that the company's first obligation was to provide it where it had been approved or was pending approval, as well as to clinical trials.

"We work diligently to get two more facilities licensed in 2020," it said.

As for the giveaways, Novartis said the intention was for a long-term commitment, with additional doses added to the program on a rolling six-month basis based on patient need and the expansion of capacity.

Novartis said AveXis had worked with an independent bioethics advisory committee to develop the programme, which it insisted was "anchored in principles of fairness, clinical need and global accessibility to best determine the equitable global distribution of a finite number of doses."

'Blinded selection'

It would not favour any child or country over another, it said.

A third party would administer "a blinded selection" every two weeks from a pool of patients proposed by their treating physicians who had been proven to be medically eligible, it said.

Novartis stressed that patients not picked in one selection round would automatically be submitted to the pool of candidates for the next selection as long as they remained medically eligible.

But the process drew criticism.

TreatSMA, a British advocacy group for the disorder, praised Novartis for trying to increase access, but it said it had "yet to be convinced that a health lottery is an appropriate way of meeting the unmet medical needs in this severe disease."

France's AFM-Telethon, which works to raise donations to find treatments for SMA and genetic disorders, went further, criticising the programme as a cruel "lottery" offered to "parents whose children afflicted with spinal muscular atrophy are condemned to death in the short term."

"How can one envisage even for a second that the life of a child can be the big prize in a lottery," the organisation asked on its website.

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