

Article: [Scientists Designed a Drug for Just One Patient. Her Name Is Mila](#) - Gina Kolata

The article discusses how an expensive new drug was created to treat just one patient.

Please discuss if this new drug has pushed the bounds of personalized medicine and does it raise unexplored regulatory and ethical questions?

To keep your personal views private, please take two opposing positions in response to this question.

It is a dramatic story and very emotional:

- It is heart breaking to see children exposed to painful diseases and the fact that Ms. Vitarello was able to raise significant funding proved that a large number of people thought the research needed to be pursued.
- One can argue that “n-of-one” drugs are not first priority when for example neurodegenerative diseases like Alzheimer’s disease affected more than 6 million Americans in 2021.
- However, progress in research is not a linear process, in this specific case, it seems that Doctor Yu’s team had a breakthrough which might not always easy to replicate for other rare diseases.
- All the research and financial cost seem to have been wasted knowing that Mila passed away on February 11, 2021. At the same time, what was learned in term of research, technical methodologies, financial contribution set up, drug testing, team coordination, governmental procedures to put in place, were a valuable experience which could be leveraged for other rare diseases.
- The treatment was approved by the FDA which has for main mission to protect public health and have experts who are the most equipped to consider immediate and long-term consequences of allowing these types of research.
- There are been several case where medical interventions for the many has failed particularly for diseases having genetic origins. This story is the epitome of personalized drug, in the future some therapies will be designed to be unique for every patient and it is already a reality for example in prostate cancer in which a custom-tailored treatment is designed based on patient genomic fingerprinting.
- For question of urgency, the drug toxicity testing was rather short compared to usual longitudinal clinical studies which can take years to reach any conclusions.
- Expensive therapies like Milasen cannot be covered by the existing healthcare system, and might be affordable only to the most privileged. There must be initiatives to develop generic therapies targeting low-resource segments of our society.
- Any new drug therapy requires an incredible amount of money until better and cheaper treatments are discovered eventually leading all the way to low-cost generic treatments. To scale up medical platform for personalized drugs should be created which are flexible enough so to be repurposed for different rare diseases (heterogeneous in design).

Governmental organizations and researchers will have to reach agreements which balance the different needs for personalized medicines and the ethical guidelines to follow so as our society as a whole benefit of the progress made.