Disease/information ? orphan diseases, rare
Gaucher disease, rare, fatal, without treatment children die at 2 ; only affects about 6,000 people in the US
Symptoms ? swollen liver and spleen, fever

Treatment ? infusion in hospital through a surgically implanted port at the center of his chest
Cerezyme, liquid medicine that replaces an enzyme he lacks, approved in 1994, made by sanofi Genzyme
Cost ? high price tags, often in the 6 figures for a year of treatment, 300,000 a year
Drugs on the market ?

Patient/details *Luke Whitbeck 2 years old has Gaucher disease, calls the port in his chest imparted for his infusion : a button*
He already suffered from the disease when he was 5 months old : swollen liver and spleen
One month he had 15 days with a fever : tired, difficulties eating, lost weight
When 18 month he was tested in hospital

Issues raised in the extract (regarding drug prices or encouraging companies to develop innovative drugs). Are drug prices always justified ?
The Orphan Drug Act of 1983 lowered prices (Abbey Meyer is one of its architects + Founder of the National Organization for Rare Disorders, it gave drug companies incentives to develop drugs for small groups of people : as a result, in 2015 nearly half of the new drugs approved by the US FDA were for rare diseases.
The Orphan Drug Act gave drug makers a lot of financial incentives to ensure drugs would be made even if they didn't make a profit : a fee waiver, tax credits + acces to special grants, garantees no direct competition for 7 years
Treatments for Gaucher cost $300,000. The FDA has no authority over prices. The cost has not changed for 20 years
Sanofi spokesman says the price is 33% lower today than 22 years ago : determined by the clinical value it provides patients + the rarity of the disease + cost of R&D