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Inside The Pricing Of A \$300,000-A-Year Drug

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I cover science and medicine, and believe this is biology's century.

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Last night, NPS Pharmaceuticals announced that it was pricing Gattex, its drug for short bowel syndrome, at \$295,000 per patient per year, about triple what analysts on Wall Street expected. It is the fourth drug

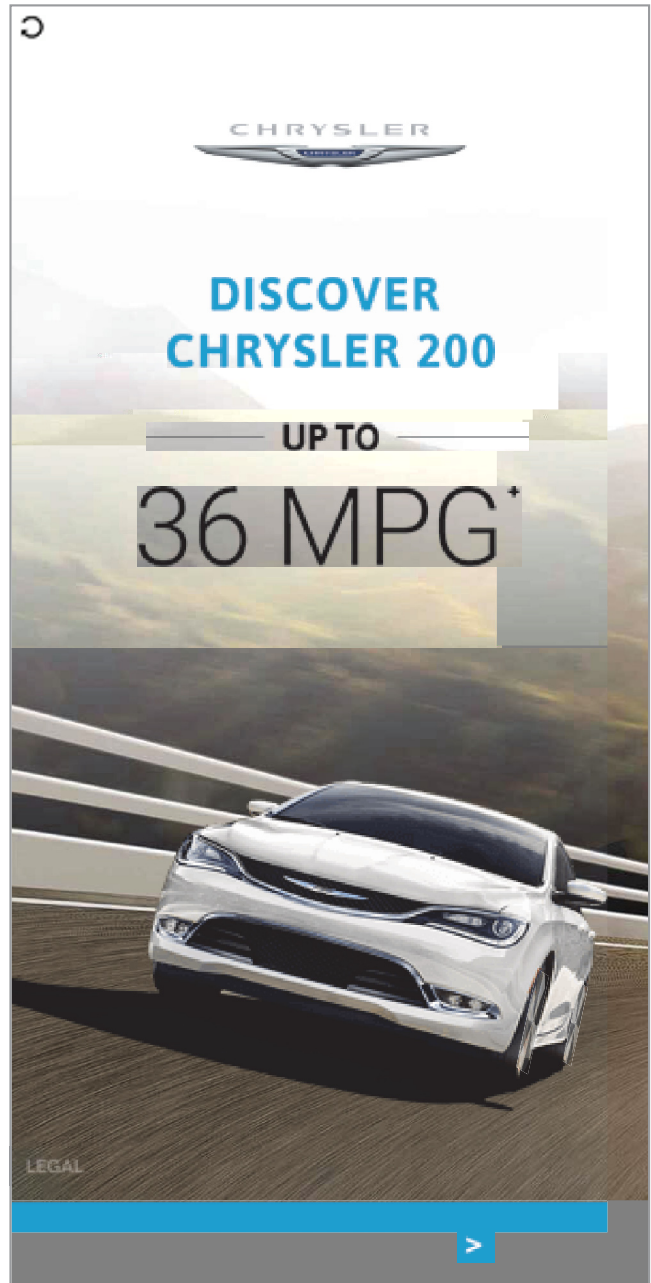
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than \$200,000 per patient per year. The others are: Kalydeco for cystic fibrosis (maker :Vertex Pharmaceuticals); Elelyso for Gaucher’s disease (Protalix and Pfizer); and Juxtapid for homozygous familial hypercholesterolemia (Aegerion Pharmaceuticals.) That represents 10% of the drugs approved by the Food and Drug Administration last year and a 44% increase in the number of such high-priced rare disease drugs on the market.

Francois Nader, the chief executive of NPS Pharmaceuticals, spoke with me this morning about how he and his team priced Gattex. Here is a roadmap for how a medicine can be priced at an annual cost equivalent to a house. These bullet points reflect NPS’ reasons for pricing the medicine as it did.



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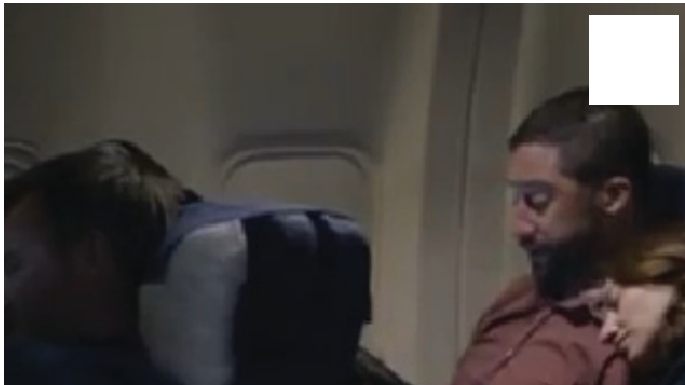
1. **Medical value:** Gattex is the first non-surgical treatment for short bowel syndrome, a disease which requires patients to get their nutrition parenterally or through a vein. A sixth

weaned from this parenteral nutrition. “this is a product that significantly improves the daily living of these patients,” says Nader. “They feel liberated and for them it is a life-changing event.”

2. **Direct costs:** Parenteral nutrition can on its own cost \$100,000 a year. But Nader says there are other costs that can be as high as \$500,000 per patient, and that Gattex is expected to reduce those costs, which include hospitalization, as well, although that has not been shown in clinical studies. Short bowel syndrome also leads to increased mortality.



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2. **Indirect costs:** Gattex improves

patients to return to work, so that they no longer receive disability payments and become productive members of society.

4. **The orphan drug premium:** NPS found that there may be as few as 3,000 short bowel syndrome patients in the country, fewer than expected, after it polled companies that provide parenteral nutrition. Drugs for rare diseases cost more. “Until recently orphan products were called neglected diseases. I think that society and payers are willing to pay a premium for these diseases that were ignored.”
5. **Will insurers pay?** NPS says it polled insurers, patients, and doctors about what they would be willing to pay, and Nader says that they would have been able to pay a price even higher than what it is charging. Nader says that if the existing short-bowel treatment, Zorbitive, were used on an annual basis, it would cost \$290,000 per year too.

How, though, does anyone pay for such a medicine? The answer is that pricing of these rare disease drugs, known as ultra-orphan drugs in the biotech industry, are not paid for the way that most medicines are. Nobody buys them out of pocket, and the manufacturer refuses to grant discounts to insurers or to Medicare. Commercial insurers will usually not pay the whole cost of the drug, asking patients to cover a co-pay that could

what is known as a co-pay assistance program, NPS will cover patients' out-of-pocket costs. Nader thinks 51% of potential Gattex patients are on commercial insurance.

Another 34% of potential Gattex patients are on Medicare. For these patients, rare disease organizations will help pay the bill. NPS will give unrestricted grants to those rare disease organizations, but can't say what they'll be used for. Another 15% of patients probably won't be able to pay at all. Those patients will get the medicine for free. NPS will have a field force of 38 people who will market the drug and get to know each patient personally and to negotiate insurance reimbursement for each drug. The general outline of this plan is similar to what is done by other rare disease companies like Alexion Pharmaceuticals, Sanofi's Genzyme unit, and BioMarin. The strategy of targeting these rare diseases has been one of the most successful in biotech over the past decade; it has, for instance, made Alexion into one of the industry's biggest stars. Nader says it cost about \$250 million to develop Gattex, which is not that expensive in an industry where a drug can chew up \$1 billion or more in development costs before getting approved.