DNDi
Drugs for Neglected Diseases *initiative*

Fondation ARPE
Monsieur Stanislas Poniatowski, Président
Madame Madame Julie Vaisy, membre du conseil de Fondation
Stockerstrasse 23
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Genève, le 11 septembre 2017

Monsieur le Président de la Fondation ARPE,
Chère Madame,
Chers membres du Conseil de la Fondation ARPE,

Votre don du 17 mars 2017 nous est bien parvenu, nous vous prions de bien vouloir nous excuser du retard avec lequel cette lettre de remerciement vous parvient. Un retard inversement proportionnel à notre reconnaissance.

Votre soutien régulier nous permet de continuer notre engagement de recherche et de développement de traitements en faveur des patients infectés par le virus de l'hépatite C. Dans mon précédent courrier au mois de juillet, je vous ai fait part de l'évolution du projet, permettez-moi aujourd'hui de vous adresser un article paru mi-juillet dans le New York Times. J'espère que vous aurez de l'intérêt à le parcourir, et surtout n'hésitez pas à me contacter si vous voulez en discuter.

J'aimerais aussi vous proposer d'organiser, si vous le désirez, une rencontre à Genève, Paris ou Zürich au cours de laquelle nous pourrions ensemble exposer à votre entourage notre engagement commun. Dans cette perspective, serait-il possible de nous revoir au cours du dernier trimestre 2017 ?; je pourrais aussi à cette occasion vous faire part des activités en cours et des perspectives à venir.

Dans l'attente de vos nouvelles, je vous prie de croire, Cher Monsieur, Chère Madame, Chers membres du Conseil de la Fondation ARPE, à l'expression de mes respectueux et chaleureux messages.

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Annexes: Note de crédit don du 17 mars 2017 & article New York Times 18 juillet 2017

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The New York Times

Escaping Big Pharma's Pricing With Patent-Free Drugs

Fran Quigley

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How's this for a great deal? The United States government funded research and development of a new vaccine against Zika. But the Army, which paid a French pharmaceutical manufacturer for its development, is planning to grant exclusive rights to the vaccine to the manufacturer, Sanofi Pasteur, along with paying Sanofi up to $173 million.

A demonstration of a new malaria treatment at Fundacao Oswaldo Cruz, in Rio de Janeiro in 2008.
Credit: Ricardo Moraes/Associated Press

Sanofi will be free to charge the United States American health care providers and patients any price it wishes. Although American tax dollars funded the vaccine, and the United States took the economic risks, history suggests that many Americans would not be able to afford it.

This is a negotiating strategy of unconditional surrender. Although President Trump said before taking office that drug companies were "getting away with murder" and had campaigned on lowering drug prices, his administration is doing the opposite. A draft order on drug pricing that became public in June would grant pharmaceutical companies even more power to charge exorbitantly. For example, it could shrink a federal program that requires companies to sell at a discount to clinics and hospitals serving low-income patients.

Exorbitant prices are one thing that's very wrong with the way we make medicines. The other is: medicines for what? If a malady has no market in wealthy countries, it gets no attention. Poor-country diseases, known as "neglected diseases," have a ferocious impact: One of every six people in the world, including a half-billion children, suffers from neglected diseases. Yet of the 756 new drugs approved between 2001 and 2011, less than 4 percent targeted those diseases. The industry spends far more on lobbying government agencies to extend monopolies on high-cost drugs — or hand out deals like the Zika vaccine — than it does on research for a vaccine against dengue fever, which poses a risk for 40 percent of the world's population.

But there's one drug company that behaves differently.

When Bernard Pécoul was a young physician working for Médecins Sans Frontières/Doctors Without Borders in the 1980s and 1990s, the only available treatment for Human African Trypanosomiasis, better known as sleeping sickness, horrified him.

The protocol called for multiple, extremely painful, injections of melarsoprol, an arsenic-based compound. People in the Democratic Republic of Congo called it "fire in the veins." Children who received it had to first be forcibly restrained. The treatment was so toxic that it caused fatal brain swelling in 5 percent of the patients, and it didn't work for a third of those who survived it. But sleeping sickness is fatal if not treated, so there was no choice.

What outraged Pécoul, who is French, was that melarsoprol had been around since 1949, and nothing better had been developed since. To the physicians at Doctors Without Borders, it was just one example of the lack of effective remedies for the diseases of the poor. "We were well aware of the difference between the quality of treatment we were providing and the care that was available to patients in high-income countries," Pécoul said.

Bernard Pécoul at the DNDi office in Geneva.

In 2014, the Ebola virus spread in epidemic proportions while physicians had no response. Promising vaccines to prevent Ebola, and drugs to treat it, had been in early development years before. But those medicines were allowed to languish without completion to public availability because pharmaceutical companies saw no prospect of significant profit to be made from their sale. Ebola claimed 11,000 lives, demonstrating in stark terms the core limitation of the current medicines research and development model.

Pécoul and Doctors Without Borders decided to tackle the diseases that were killing the global poor. Doctors Without Borders dedicated its 1999 Nobel Peace Prize award money to providing seed funding for the Drugs for Neglected Disease Initiative, known as D.N.D.I. The aim was to see what could be accomplished when research priorities ignore questions of profitability, and the price of medicines is "delinked" from research costs, which are instead shouldered by public financing or philanthropy.

An immediate challenge was that D.N.D.I. possessed none of the required hardware for the expensive drug research and development process: It had no labs, no manufacturing facilities, and no distribution process. It fell to Pécoul to recruit partners, including private pharmaceutical companies he persuaded to share drug compounds that had been uncovered but abandoned because of lack of profitability. Government and grant funding allowed D.N.D.I. to pay existing labs to test promising drug candidates and facilities to manufacture the end results. D.N.D.I. itself took on the difficult task of conducting clinical trials in the rugged, remote areas where neglected disease are most deadly. Pécoul says the multi-partner approach is akin to being the "conductor of a virtual orchestra."

D.N.D.I. has already delivered seven new patent-free, low-cost treatments for neglected diseases. A partnership with Sanofi (yes, the same company) led to the distribution of a fast-release oral antimalarial treatment to nearly 500 million adults and children, at a cost of less than a dollar per patient. Dr. François Bompart, vice president for access to medicines at Sanofi, said that Pécoul was not shy about insisting that the company had a moral obligation to make its products widely available. "Bernard is able to combine the role of a sometimes aggressive challenger with a personal style that is likable," Bompart says. "He is offended by situations that are unfair, but he has established D.N.D.I. as a credible partner with the private sector."

Last month, Tina Rosenberg wrote about the Center for Epidemic Preparedness Innovations, which is raising foundation and public money to develop vaccines for dangerous pathogens. C.E.P.I. is just for vaccines. D.N.D.I. also works on treatments. It tries to improve existing medicines to make them easier to use in the field: for example, administered orally.

And it develops new drugs; the more than 30 projects in its current pipeline include 15 entirely new chemical entities.

To create seven drugs, with 30 more in testing, D.N.D.I. has spent $290 million to date. For-profit pharmaceutical manufacturers claim that it costs them $2.5 billion to develop a single drug, a figure often derided by critics as a wild exaggeration.

Another way D.N.D.I. is different is that it makes its research available for follow-up studies, adhering to an open-source philosophy that is unusual in patent-focused biomedical circles. It uses the term "public goods" to describe its products, harkening back to an era when medicines were widely considered to be off-limits to patenting and the resulting monopoly price markups. As Jonas Salk famously said when asked why he did not

D.N.D.I. does not claim it can replace the for-profit pharmaceutical industry. "Every single drug that the (D.N.D.I.) initiative is developing — and they're all worthwhile — piggybacks on other people's quest for profit," a pharmaceutical researcher, Derek Lowe, wrote in a Science magazine blog post last year.

But clearly there's room for more nonprofit drug companies. "D.N.D.I.'s success is ample proof that the model of nonprofit, open-source research and development is a viable alternative to exclusive reliance on patent and data monopolies," said Brook Baker, a professor at Northeastern University School of Law who studies pharmaceutical development issues. Recent reports on the crisis of access to medicines by the United Nations and the British medical journal The Lancet single out D.N.D.I. as evidence that different approaches to research and development can provide improved efficiency and affordability.

Pécoul and his D.N.D.I. colleagues say their biggest challenge now is securing sustainable funding for research — another illustration of the limits of a model with no profits to invest in research. But the government funds research all the time — it's just often turned over to for-profit companies. The Zika vaccine is one example. The prostate cancer drug pacilataxel, the leukemia medicine imatinib and many mental health and H.I.V. medicines and vaccines can all trace their origins to government-funded research — only to be handed over to industry to charge what they want.

"For-profit pharma has depended upon the National Institutes of Health and other government programs to fund all steps of research and development," said James Love, director of Knowledge Ecology International, which is helping to lead the opposition to the Zika vaccine deal. "Governments should provide more resources and more opportunities for nonprofit companies to bring drugs and vaccines to market." That way their products would be available and affordable to all.

Some economists have argued that since Medicare and Medicaid are the leading purchasers of drugs (and Medicare is forbidden by law from negotiating on price), the money saved by buying from nonprofit drugmakers could easily replace all privately funded research and development. The increased public research dollars could then be applied, D.N.D.I.-like, to develop the medicines that would have the most significant public health impact.

This would help rich countries as well as poor ones. D.N.D.I. has already received funds to work on treatments for hepatitis C. Cures exist, but companies charge as much as $84,000 for a typical 12-week course of treatment. This means that most people with Hep C cannot get the drugs.

In the meantime, D.N.D.I. has made progress in tackling the disease that helped spur its creation. In 2009, it started the first new sleeping sickness therapy in 25 years, a combination treatment that avoids the toxicity of melarsoprol and reduces the number of intravenous injections that made other options impossible to use in remote settings. Since humans are the main reservoir of the disease, increased treatment has caused sleeping sickness cases to drop to just a few thousand a year. And D.N.D.I.'s pipeline of projects under development includes its gold standard of a single-dose oral medicine for sleeping sickness, a prospect that is causing health experts to envision the global elimination of the disease.

"I would not say we are the only solution, but we do show a different way that can work," Pécoul said. "We cannot depend on a for-profit model to address the needs of all patients — that has been well-demonstrated."

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