

a year ago, in a *JAMA* editorial (310, 1343–1344, 2013), about Kalydeco's high price and the profits flowing back to Vertex and to CFF in royalties. This deal amplifies the debate.

Beall agrees that Kalydeco's price is too high. He worries, like many others, about the sustainability of this and other expensive treatments. "We'd gladly have sacrificed our royalties for a lower price," he claims. But "we weren't at the pricing table, and probably wouldn't have influenced it if we were," he continues. At least now, by monetizing the royalties, we can "take some of those revenues and put them back towards helping patients."

CFF's mission is far broader than supporting R&D. It runs and coordinates clinical trials and a patient registry across dozens of care centers, offers patient assistance programs and reimbursement

advice and retains a small interest in a specialty cystic fibrosis pharmacy set up in 1998 (majority ownership was sold to Walgreens in 2012). Beall claims that as well as engaging with insurance companies to promote access, the foundation also encourages Vertex to sustain its co-pay assistance programs to allow those patients on lower incomes to obtain the drug. The vast majority of the first 1,000 patients taking Kalydeco "have been able to get the drug at a reasonable price," he asserts.

Drug pricing aside, with the US National Institutes of Health's funding stagnating, venture philanthropists such as CFF have become an increasingly important source of investment and other forms of support for early-stage drug discovery. Yet as venture capitalists and pharma companies increasingly seek less risky, later-stage assets, "this creates a greater demand to advance projects even further to attract their attention and investment," concedes JDRF's Insel.

Which isn't to say exciting, early-stage ideas are ignored: many venture philanthropists are pursuing alternative models, such as spin-outs, to help these find an outlet. FasterCures is trying to facilitate dialog between universities and nonprofits, includ-

ing establishing some standards for termsheets.

And CFF, newly flush with cash, now has the resources to take even more risk. "If we need to seed a technology, we will," says Beall, enthusing about gene repair, oligotide/nucleotide delivery programs and mRNA therapies as the next-generation of potential treatments. The foundation plans to expand its laboratories in Boston to create an academic consortium, including leading institutions, as well as companies, with complementary technologies and expertise. To this end, headcount at CFF's existing laboratory, set up to help companies run assays, de-risk their programs and provide

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other resources will be expanded by 50% in the next six months to include stem cell and gene-editing resources. The ultimate goal of the newly formed

consortium: to create a one-time, lifetime therapy for cystic fibrosis patients.

For Alzheimer's Foundation's Fillit, far from raising concerns over the role of nonprofits in drug development, the CFF deal "shows the value of philanthropy in helping bring drugs to market. It confirms for philanthropy that seeking a return is an important way to do business."

And this deal makes it clear, if it wasn't before, that nonprofits are run as businesses—and will be held to account as such. They may not be seeking the same returns as for-profits, but they're still seeking return nowadays, to support their mission and invest their donors' funds appropriately. Not all CFF's investments have worked out (denufosol, for instance, from Durham, North Carolina-based Inspire Pharmaceuticals). But many have. Life expectancy for cystic fibrosis sufferers has more than doubled over the last 30 years. Thus, there's little reason CFF won't continue to deploy its money strategically and effectively. Will this windfall dampen future fundraising? "It's too early to judge," says Beall. "But," he adds, "we just got another half-a-million-dollar gift this week. People like to invest in winners."

Melanie Senior London

FDA OKs breakthrough B meningitis vaccine

The US Food and Drug Administration (FDA) has granted Pfizer subsidiary Wyeth Pharmaceuticals approval of its Trumenba vaccine for the prevention of meningococcal disease caused by *Neisseria meningitidis* serogroup B in people ages 10 to 25, the first of its kind in the US. Previously the only meningococcal vaccines licensed in the US covered four of the five main disease-causing serogroups: A, C, Y and W. Another serogroup B vaccine, Novartis' Bexsero, was approved by the EU in January 2013 and is available in 34 countries. Trumenba was approved less than six months after Wyeth's submission under the FDA's breakthrough therapy program.

Universities, drug makers form GPCR Consortium

Chinese and US universities have joined a trio of biopharma companies in an open-source research collaboration to map the known G protein-coupled receptors (GPCRs) using the latest imaging technology. ShanghaiTech University's iHuman Institute, the Shanghai Institute of Materia Medica, the University of Southern California, Amgen, Sanofi and Ono Pharmaceutical make up the nonprofit GPCR Consortium; the collaborators hope to bring in additional partners to plot an initial 200 of the 826 known GPCRs, with a view to applying the resulting discoveries to the treatment of diabetes, cancer and mental disorders. The consortium plans to publish its findings in the public domain.

BMS options Galecto buy

Bristol-Myers Squibb has signed an exclusive option to buy Copenhagen-based Galecto Biotech in a deal worth up to \$444 million including an option fee, option exercise payment and potential milestone fees. The acquisition depends on the early-stage trial results of Galecto's lead candidate TD139, a potent, specific inhibitor of the galactoside-binding pocket of galectin-3, enabling direct targeting of the fibrotic tissue in the lungs through inhalation, while minimizing systemic exposure. The compound would complement BMS' fibrosis portfolio, which includes a lysophosphatidic acid 1 receptor antagonist being developed to treat idiopathic pulmonary fibrosis.

“Since 2002 we got 71 new drugs against advanced cancer. Average lifespan increase they give? Two months.”

In a blog post on modern medicine, Richard Lehman, a British physician, posits that expensive cancer therapies that serve small patient populations do not serve the public well. (*BMJ Blog*, 18 December 2014)