IN brief

Pharma bankrolls academic institutes

This spring, Merck and Bristol-Myers Squibb became the latest big pharma suitors to forge a fresh round of partnerships with academia. Merck of Whitehouse Station, New Jersey, announced that it would pony up $90 million over seven years to help fund the California Institute for Biomedical Research (Calibr)—a new nonprofit based in San Diego. Calibr will hire a dedicated staff whose mission will be to bring early-stage research through preclinical development. Researchers from any institution will be able to approach Calibr to further develop their research. Although Merck retains the option to license exclusively any drug candidate developed at Calibr, the pharma giant will have no control over the institute's research agenda, nor will Merck employees have joint posts at Calibr, says Jim Schaeffer, executive director of licensing & external research for Merck Research Laboratories. Calibr's nonprofit status will also ensure that bureaucratic wrangling over intellectual property rights will be minimal, Schaeffer adds. Also in March, Bristol-Myers Squibb of New York announced that it was strengthening its partnership with Duke Translational Medicine Institute in Durham, North Carolina. A new joint steering committee will identify areas of common interest and greater opportunities to collaborate. The partnership involves very little money and intellectual property issues will be negotiated on a case-by-case basis. In April, Roche announced it will also be bolstering its partnership with the French National Agency for Research on AIDS and Viral Hepatitis, and Baylor Research Institute in Dallas, with a licensing agreement to bring vaccines targeting dendritic cells to treat HIV and hepatitis infections into clinical development (financial details were not disclosed). Inserm Transfert, the French National Institute of Health and Medical Research's knowledge transfer company, will manage intellectual property. The announcements follow on the heels of Johnson & Johnson's January launch of the Janssen Labs, the company's 30,000-square-foot innovation center in the Torrey Pines Mesa area near San Diego. In late March, the nearby University of California (UC) San Diego agreed to build (*Nat. Biotechnol.* **4**, 297, 2012) a $110-million facility called the Center for Innovative Therapeutics that will provide laboratory space for UC San Diego Moores Cancer Center researchers and biotech companies, and focus on translational cancer research. Although the recent wave of partnerships clearly reflects industry's need to find new ways to tap innovation, they may also be "a natural consequence" of academic centers having placed more emphasis on translational research, says Gerard Zurawski, principal investigator at Baylor. "Partnering with academic institutions may have simply become more attractive to industry." *Gunjan Sinha*

Agency defies advice and rejects gene therapy for third time

For the third time, the European Medicines Agency (EMA) recommended that Glybera, a treatment for the inherited disorder lipoprotein lipase deficiency (LPL), should not be approved. The refusal on April 20 means there's still no approved gene therapy in a regulated Western market. It was bad news for suffers of the ultra-rare disorder and amplified the fault lines that the review of the product has opened up, both between different expert committees of the EMA, and between the agency and its masters at the European Commission.

The EMA in London gives a thumbs down to AMT's gene therapy in May.

Indeed, EMA's Committee for Medicinal Products for Human Use (CHMP) only got to vote on the product for a third time because in January the commission refused to rubber stamp an earlier recommendation to reject Glybera (alipogene tiparvovec), taking the unprecedented step of telling the CHMP to think again.

Glybera was not turned down because of any concerns about the safety of using an adeno-associated viral vector to deliver a correct copy of the aberrant gene, but because the CHMP concluded the developer, Amsterdam Molecular Therapeutics (AMT), had not demonstrated Glybera's efficacy in reducing attacks of acute pancreatitis that are the hallmark of LPL. The decision to reject Glybera could not have been closer. An absolute majority of the 32-member-strong committee—17 votes—was needed for approval. But on the day the CHMP voted 16-15 in favor one member was absent, and Glybera got the thumbs down.

Not only was the CHMP's decision to reject Glybera balanced on a knife edge, it was also contrary to advice received from the EMA's Committee for Advanced Therapies (CAT), a body set up specifically to provide guidance to the CHMP on gene, cell and tissue therapies. After convening an expert group to evaluate the clinical data and the science underpinning the product, CAT recommended Glybera should be approved under exceptional circumstances that would strictly limit the situations in which the one-off treatment is used, but allow efficacy data to accumulate.

Anyone with any common sense can see the drug is fit to be approved," said Jörn Aldag, CEO of AMT. That it hasn't been is "purely bureaucratic," he claims. AMT, of Amsterdam, has now been taken private, transferring its gene therapy assets to a new company, uniQure BV, and there will be no further investment in Glybera.

Alastair Kent, one of Europe's leading representatives of rare diseases patients' groups, agrees the decision is "perverse" and says the CHMP "should not be allowed" to ignore the advice of CAT, which is the committee "best qualified" to judge the quality, safety and efficacy of advanced therapies.

Kent, who is head of Genetic Alliance UK and president of the European Genetic Alliances' Network, has written to the European Commission to protest the decision."

In the case of Glybera, the CHMP was weighing data from only 12 patients when it concluded the reduced incidence of pancreatitis could have been due to other factors "such as changes in lifestyle and diet, and the natural course of the disease."

As Aldag notes, patients have indeed changed their diets because without treatment the only way to avoid acute pancreatitis is to maintain a zero-fat diet. He believes that with so few patients affected by such an insidious disease, individual case histories should be allowed to count. For example, although a fat-free diet is obviously at odds with pregnancy, one woman treated with Glybera increased her fat intake and carried a baby to term without suffering a single attack of pancreatitis.

For Kent, the decision to reject Glybera is not only bad in itself, it is likely also to have important consequences for the development of therapies for rare diseases. The regulatory process alone cost AMT €15 ($19) million of a €50 ($63) million total investment in Glybera. "The difficulties in generating evidence to support approval of treatments for very rare conditions are well demonstrated. If the CHMP insists on setting the bar unreasonably high, investors won't take the risk," Kent says.

The European Commission has until mid-June to respond to the recommendation that Glybera be rejected. Having refused to agree once, it remains to be seen if it will now back the CHMP's opinion. **Nuala Moran** *London*