IN brief

Academia's $1,000 genome

In August, the National Human Genome Research Institute (NHGRI) awarded $14 million in grants to support technologies that will enable rapid sequencing of a human genome for $1,000 or less by 2012. Grantees include seven university-based research groups and two companies—Electron Optica of Palo Alto, California, and Stratos Genomics of Seattle—that will undertake studies of new platforms and follow-on studies. Since 2004, NHGRI, part of the National Institutes of Health, has been spending between $18 and $22 million each year to bolster next-generation sequencing technologies, says Jeffery Schloss, program director of Technology Development Coordination at NHGRI. The goal is to ensure that genome sequencing will be easily available to researchers and healthcare providers. Technology supported by NHGRI has already brought the price down below $20,000, "but the data aren't yet of the quality we'd like to see," says Schloss. "The question is whether researchers can improve on these things." Many commercially successful sequencing technologies were supported by NHGRI grants at some point during development including Roche's 454, Oxford Nanopore's SOLiD, and Helicos' tSMS, says Jay Shendure, associate professor of genome sciences at University of Washington, Seattle, and a 2011 grant recipient. "This is a great example of the NIH supporting risky, innovative work that is explicitly technology development and also an example of how those bets can pay off." *Gunjan Sinha*

China eyes Western biologics

Shenyang-based 3SBio forged a venture partnership with Taizhou Oriental to license biopharmaceuticals from Western companies for late-stage development or manufacturing in China. In August, the biotech formed 3SBio Ventures with an initial investment of RMB200 million ($31.3 million) from 3SBio and RMB50 million ($7.8 million) from Taizhou Oriental, the investment arm of an industrial park in the wealthy eastern Jiangsu province. The new fund will seek Chinese licensing rights to biologic therapeutics for use in oncology, nephrology and inflammatory diseases. The fund will allocate $3–5 million to each company, with the first investment expected this year. The 3SBio case is not unique. More than five years ago, the state-owned Beijing Pharmaceutical Group, set up a company in California to seek early-stage pharma products for further development in China. The economic downturn in the US and Europe and the booming development of the Chinese pharma sector, plus heavy government support and Chinese returnees trained in the West, have facilitated the process, according to Fang Hu, former president of Shanghai-based Sunway Bio, who is now operating a contract research organization to help small, innovative biotech firms develop clinical trials. *Hepeng Jia*

Hemophilia market awaits next-generation therapies

As Biogen Idec and Swedish Orphan Biovitrum prepare to report phase 3 data from a pivotal trial in hemophilia patients of their long-acting, recombinant version of the serum coagulation factor VIII (FVIII) next year, several next-generation hemophilia therapies are progressing in the clinic. In addition to Biogen/Biovitrum's product FVIII:Fc, a FVIII linked to the amino terminus of the Fc domain of IgG1, Recoly of Brussels, and its partner Bayer, of Leverkusen, Germany, have already filed for approval in undisclosed territories for LongAte, a liposome-formulated plasma-derived FVIII, with a recombinant-based counterpart (BAY-79-4980) in phase 2 studies. Several other companies are developing hemophilia products with half-life extension technologies, including Freising, Germany–based XL Protein, Amunix, of Mountain View, California, and Bagsværd, Denmark–based Novo Nordisk.

Daniel Mihailescu/Newscom

Water fountains in Bucharest coloured red by Romanian Hemophilia Association to draw attention to the disease. Recombinant coagulation factors control symptoms but the short duration of hemophilia products remains a major limitation.

A recent report on hemophilia treatments from London-based market research firm GlobalData concludes that recombinant FVIII and other coagulation products will soon supersede plasma-derived products and foresees that hemophilia regimens will increasingly shift from symptomatic treatments to prophylactic approaches that prevent joint bleeding. Hemophiliacs with access to these costly therapies (FVIII therapies run up to $100,000/year) can expect normal or near-normal life expectancy, particularly if they start therapy at an early age. Even so, they face considerable quality-of-life constraints. Because of the short serum half-lives of both FVIII (deficient in hemophilia A) and coagulation factor IX (deficient in hemophilia B), many patients need to take intravenous injections on alternate days. Compliance is a major issue. Moreover, the development of neutralizing antibodies—known in the hemophilia community as 'inhibitors'—can occur in up to 30% of hemophilia A cases (and in a smaller percentage of hemophilia B) and complicates management of the condition even further. There is, therefore, considerable scope for technological improvement in the field, according to analyst Ankita Ahuja, who authored the report.

In the near term, Biogen/Biovitrum's FVIII:Fc and Recoly/Bayer's LongAte look most likely to reach the hemophilia A market. FVIII:Fc has been shown to have a half-life 1.7 times longer than Deerfield, Illinois–based Baxter's Advate (recombinant human FVIII) in a phase I/IIa trial. And Recoly/Bayer claim that the improved hemostatic efficacy of LongAte enables once-a-week prophylactic treatment compared with conventional regimens where plasma-derived FVIII is injected at two- to three-day intervals.

Several other half-life extension technologies are also being explored. XL-protein is using a process it terms PASylation, involving the addition of a 600-residue unstructured (random coil structure) polypeptide chain containing proline, alanine and serine residues to FVIII. Amunix, of Mountain View, California, is working with Biogen-Idec on extended life versions of recombinant blood factors (FVIII, Factor IX and Factor VIIa) that have been fused to a long unstructured and nonrepetitive polypeptide (>100–3,000 residues in length). And Novo Nordisk is trialing a technology it acquired from former partner Neose Technologies (which ceased trading in 2008) whereby FVIII can be modified in a 'site-specific' manner with polyethylene glycol (PEG; PEGylation), leading to easier production and greater product consistency. "We do not need to modify the amino acid backbone," says Soren Erik Bjørn, vice president of hemostasis science at Novo Nordisk. It could double the half-life of FVIII. "We will investigate an infusion frequency of once every four days," he says.

Several other firms are exploring more experimental approaches that could complement classic coagulation factor therapy. Baxter has acquired a program from Cambridge, Massachusetts–based Archemix involving the development of aptamers to target tissue factor pathway inhibitor. Alnylam Pharmaceuticals, also of Cambridge, is developing RNA interference (RNAi)-based agents targeting protein C. And Thrombotargets, of Castelldefels, Spain, is developing several anti-hemorrhagic drugs against targets implicated in coagulation, such as lipidated tissue factor. These treatments are as yet unproven in terms of clinical validation of safety and efficacy. "We need to have human trials to give us a clear answer," says Rebu Ninan, center of excellence manager, oncology and blood disorders, at GlobalData. **Cormac Sheridan** *Dublin*