First moss-made drug

The world's first moss-produced drug candidate has the go-ahead to enter clinical trials. German regulators in September told Greenovation Biotech of Freiburg that it could begin a phase 1 clinical trial evaluating moss-aGal, a recombinant form of human alpha galactosidase, as an enzyme replacement treatment for Fabry disease. Like all plants, moss engage in post-translational modification of proteins, but unlike other plants, it does not make hyperglycosylated proteins that are immunogenic in humans. Greenovation's moss, *Physcomitrella patens,* produces predominantly N-mannose terminated proteins. This improves uptake by cell-surface mannose receptors throughout the body, potentially qualifying this product as a "biobetter." Moss has several other attractive features: its genome is only 511 Mbps, and it is haploid whereas other plant systems are diploid or polyploid. In addition, moss predominantly uses a homologous recombination system for repair, which makes gene replacement efforts more efficient. Several versions of alpha galactosidase are on the market or under development: Fabrazyme (agalsidase beta) from Sanofi/Genzyme of Paris, which has been in short supply since 2009, Replagal (agalsidase alfa) from San Diego's Shire, and another plant-produced (carrot and tobacco) recombinant version of this enzyme, called PRX-102, developed by Protalix BioTherapeutics of Carmiel, Israel.

Seek 1 million US citizens

The US National Institutes of Health (NIH) will start enrolling 1 million or more US citizens as the first key step to implementing President Obama's nationwide Precision Medicine Initiative (PMI) (*Nat. Biotechnol.* **33**, 325, 2015). Plans for the clinical research cohort were outlined in September by the NIH Advisory Committee to the Director. Volunteers recruited to the study will agree to let researchers collect biological specimens as well as detailed information about their health status, including genomic sequence data. In turn, that information will be systematically curated and analyzed to underpin the PMI. Among its recommendations, the working group emphasized the importance of actively engaging those individuals who join this cohort, allowing some of them to participate not only in clinical studies outside the initiative but also in oversight of the initiative itself. Giving volunteers a say in how the PMI is governed and allowing them to decide how fully they will participate once it is under way are but two of the steps recommended to build adequate safeguards, including guarantees of maintaining individual privacy, into the experience for all participants, according to NIH director Francis Collins. NIH anticipates spending $215 million in fiscal 2016 in this first phase of the initiative.

will allow the group to remain in North Carolina to pursue the possibility and grow as its research arm. Head count is expected to double in the next 12–18 months.

Phoundry is the only company, so far, to emerge from GSK in the wake of the recent layoffs. That is not surprising to Art Pappas, a former GSK employee and founder of A.M. Pappas & Associates, a Durham-based venture capital firm. Pappas, also a Phoundry co-founder, who led its first seed round of financing in August, notes, "There is always going to be a lag [in new company formation] of three months to three years." He figures GSK itself is unlikely to be funding any local startups, as their early-stage work has now moved to a campus in the Philadelphia area, and to Stevenage in the UK.

Young is also concerned that GSK's departure will leave a vacuum in Research Triangle Park and North Carolina as a whole. In the absence of a big pharma, he suspects many cast-off employees will have a hard time finding the sort of work they are trained for. Pharmacoeconomic skills, for instance, such as negotiating with insurers on pricing, is important to big organizations, but biotechs seldom need them. On the other hand, North Carolina is home to some of the world's largest contract research organizations, including Quintiles, PPD and LabCorp, and their presence could provide some job openings.

Fortunately, local venture capitalists are stepping in. Hatteras Venture Partners in Durham has earmarked part of the $150 million it expects to raise for its fifth fund, for early-stage investments in the area. Malin, a Dublin-based venture firm headed by former Elan executives, has also committed a minimum $15 million to the fund. Clay Thorp, a general partner at Hatteras, also sees opportunities arising from GSK's R&D division closures. Executives and scientists often do well after departing from there, he observes. But Thorp points out that despite the talent and infrastructure at Research Triangle Park, the area "lacks a robust, multiparty investment community." Some funds that were active in local early-stage deals in the past are less so of late, he notes. "And we don't have a Biogen or a Genentech" or other large established biotech company attracting and generating research talent and deal flow, he notes.

The venture investment company is mainly attracted to the research coming out of nearby institutions like Duke University, Wake Forest University and University of North Carolina. Indeed, from 2010 to 2014, North Carolina alone received $3.1 billion in US National Institutes of Health grants, a rough indicator of the high-quality academic research projects conducted there.

Today's 60,000 jobs in life sciences in North Carolina pay twice the average salary of the overall private sector in the state, averaging in excess of $81,000. In total, some 260,000 jobs depend on the life-sciences industry directly and indirectly, says Doug Edgeton, president and CEO of the North Carolina Biotechnology Center (NCBiotech). The Center was funded as a not-for-profit entity outside of state government in 1984, and this independence has allowed it to be nimble with decisions of how to help various players with grants, loans and company recruitment. NCBiotech struggled after cuts but this year saw its $13.6 million funding restored. Although GSK's participation in early-stage R&D will be missed, the North Carolina biotech scene has enough players to continue to evolve.

Deborah Erickson *New York*

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A seven-month-old GSK spin-out from the North Carolina area, Phoundry Pharmaceuticals, was acquired by Intarcia in September. Intarcia will add Phoundry's peptides to its mini-pump as a combined medication for diabetes.

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