Translational tidbits

By Lev Osherovich and Kai-Jye Lou, Senior Writers

Berg's big data dive

Berg Pharma LLC is seeking to validate its Interrogative Biology toolkit by striking academic partnerships with the **University of Miami Miller School of Medicine** and **Medical University of South Carolina**. Berg will use tissue banks and patient data from the medical schools to discover biomarkers and targets for cancer and cardiovascular, CNS, metabolic and autoimmune diseases.

The platform combines a patient's molecular data with clinical records and demographic data. The goal of the systems biology approach is to help doctors select treatment regimens and help insurance companies develop formularies.

The key, said Berg president, cofounder and CTO Niven Narain, is getting access. "We want to partner with leading laboratories with exceptional access to tissue samples and databases," he said.

The University of Miami deal gives Berg access to tissue samples and clinical data from hundreds of patients with a variety of illnesses. Berg will perform genomic, metabolomic and proteomic analyses; crunch the data in

search of predictive biomarkers and potential targets; and work with clinical researchers to validate interesting hits.

Narain said that the partnership broadens an existing deal with the laboratory of Pascal Goldschmidt, dean and SVP for medical affairs at the Miller School of Medicine. That project has yielded a prognostic marker for heart failure that Narain expects will enter clinical testing in 18–24 months.

Narain noted that Berg is also collaborating with the University of Miami to test the university's dendrimer-based drug delivery technology in preclinical disease models.

The Medical University of South Carolina deal is narrower. It is focused on discovering markers for lupus in African-American women, a high-risk but understudied patient population.

Berg will analyze samples and clinical records from a collection maintained by professor of medicine and microbiology Gary Gilkeson.

"We have a very well-characterized chronological database of patients with matched kidney biopsies," said Gilkeson. "We have a large African-American cohort in this group. A lot of research genetics has focused on Asians and Caucasians, but we don't know much about the causes of lupus in African Americans."

He said that the study with Berg initially will include 70 patients and 70 controls matched for demographics. "We have serum and urine samples. We will provide the samples to Berg, and they will then run this on their data platform, putting the results into diagrams," said Gilkeson. "Once we get the results, we will need to confirm them using other samples and to follow up on the pathways to see if they make biologic sense. We can test some of these ideas in animal models or against data sets from other demographic groups."

Table 1. Selected public-private partnerships for April 2014. In April, two new consortia—PDE4NPD and SYMPATH—received a total of about €13.8 million (\$19 million) from the EU's Seventh Framework Programme, and **The Scripps Research Institute** launched **Scripps Advance LLC** to house assets and IP related to discrete early stage research projects.

Source: *BioCentury archives*

Companies	Institutions	Business area	Disclosed value	Purpose
Diaxohit (Euronext:ALEHT); InnaVirVax S.A.	Institut National de la Santé et de la Recherche Médicale (INSERM)	Infectious disease; diagnostics	€8 million (\$11.1 million)	PROTHEVIH project to develop HIV vaccine VAC-3S, companion diagnostic test CO-3S and prognostic test DIAG-3S
Iota Pharmaceuticals Ltd. ; Top Institute Pharma ; European ScreeningPort	Oswaldo Cruz Foundation ; Spanish National Research Council ; Theodor Bilharz Research Institute ; University of Antwerp ; University of Glasgow ; University of Kent ; VU University Amsterdam ; European Commission	Infectious disease	€7.8 million (\$10.7 million)	PDE4NPD consortium to discover new molecules with drug-like properties against parasitic phosphodiesterases
Roslin Cells Ltd.	Cell Therapy Catapult ; Irish Blood Transfusion Service ; Loughborough University ; NHS Blood and Transplant ; Scottish National Blood Transfusion Service ; University of Glasgow ; The University of Edinburgh ; Wellcome Trust	Hematology	£5 million (\$8.4 million)	BloodPharma consortium to generate red blood cells from pluripotent stem cells for transfusions
Affiris AG ; Biolution GmbH ; Prosenex Ambulatorium Betriebs GmbH	French Clinical Research Infrastructure Network ; Juelich Research Center GmbH ; Medical University Innsbruck ; University Hospital Bordeaux ; University Hospital Toulouse ; European Commission	Neurology	€6 million (\$8.3 million)	SYMPATH consortium to develop two vaccine candidates from Affiris against Parkinson's disease (PD) and multiple system atrophy (MSA)

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Table 1. Selected public-private partnerships for April 2014. (continued)

Companies	Institutions	Business area	Disclosed value	Purpose
UCB Group (Euronext:UCB)	Weill Cornell Medical College	Musculoskeletal disease; Endocrine/ metabolic disease; genomics	\$8 million	Advance the college's translational research programs in bone disorders, metabolic disease and rare genetic variant analysis
Berg Pharma LLC	University of Miami Miller School of Medicine	Cancer; neurology; cardiovascular disease; endocrine/ metabolic disease	Undisclosed	Discover and develop treatments for cancer and CNS, cardiovascular and metabolic diseases
Daiichi Sankyo Co. Ltd. (Tokyo:4568)	University of California, San Francisco	Neurology; diagnostics	Undisclosed	Discover and develop new drugs and diagnostics for multiple neurodegenerative diseases
Eli Lilly and Co. (NYSE:LLY)	Unitio	Endocrine/ metabolic disease	Unavailable	Use real-world, patient-centric data to identify ways to improve care and advance outcomes in type 1 diabetes
Epigenetix Inc.	Neomed Institute	Cancer	Undisclosed	Develop bromodomain containing 4 (BRD4) inhibitors as epigenetic modulators to treat cancer
Ferring Pharmaceuticals A/S; Merck & Co. Inc. (NYSE:MRK)	World Health Organization	Hematology	Unavailable	Develop a new formulation of Ferring's carbetocin to treat postpartum hemorrhage that is designed to be stable at room temperature
GlaxoSmithKline plc (LSE:GSK; NYSE:GSK)	The University of Texas MD Anderson Cancer Center	Cancer	Undisclosed	Develop immunotherapies for cancer under MD Anderson's Moon Shots Program
Johnson & Johnson (NYSE:JNJ); Scripps Advance	Scripps Research Institute	Other	Undisclosed	Identify and invest in early stage biomedical research projects
Merck KGaA (Xetra:MRK); Pfizer Inc. (NYSE:PFE)	Broad Institute of MIT and Harvard	Autoimmune disease	Unavailable	Identify biomarkers to better define target patient populations for systemic lupus erythematosus (SLE) and lupus nephritis
Sinovac Biotech Ltd. (NASDAQ:SVA)	Institute for Translational Vaccinology; World Health Organization	Infectious disease	Undisclosed	Develop Sabin inactivated polio vaccine under the public-private Global Polio Eradication Initiative

Gilkeson said that the combination of genetic and biochemical analyses offered by Berg will provide a comprehensive picture of lupus progression.

"Putting all that together has never been done before, mainly because nobody has the money," said Gilkeson. "You can't get funding from NIH for a non-hypothesis driven fishing expedition like this."

In both deals, Berg has right of first negotiation to license new IP and will share resulting royalties with its academic partners.

Closing the gap with iPS cells

Researchers at **The New York Stem Cell Foundation** (NYSCF) have reported a key technical achievement that brings somatic cell nuclear transfer (SCNT) technologies closer in line with those used to generate induced pluripotent stem (iPS) cells.¹ The new approach could enable the translation of SCNT technologies to the cell-replacement field.

SCNT is used to generate embryonic stem cells (ESCs), but the technology has lagged behind the technologies used to generate iPS cells. The latter are generally more scalable and subject to fewer funding restrictions and ethical considerations.

However, one area in which SCNT could have an edge over iPS cell technologies is in developing cell-replacement therapies. Cells derived from ESCs should have fewer safety risks than those derived from iPS cells because the reprogramming factors used to generate iPS cells have the potential to introduce genetic abnormalities.

In a study published in *Nature*, a group led by Dieter Egli has now published an SCNT-based protocol for generating ESC lines that worked with fibroblasts from a 32-year-old female with type 1 diabetes. The resulting ESC lines could be differentiated into insulin-producing pancreatic cells as well as neuronal cells.

"Because the biology of human oocytes is poorly understood, and protocols established in animals could not be directly translated to human oocytes, the derivation of stem cells [with SCNT] using adult somatic cells is a very large technical step," said Egli, a senior research fellow at the NYSCF. "With these results, we are now more on par with that which has been achieved with iPS cells."

Other groups have published SCNT-based protocols for generating ESC lines from fetal human somatic cells^{2,3} and more recently with somatic cells from a healthy adult.⁴ However, Egli said that using somatic cells from an adult with a disease that

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**—Dieter Egli,
The New York Stem Cell Foundation**

could be treated with an organ or tissue transplant is a key technical step toward realizing potential therapeutic applications with the technology.

Islet and pancreas transplantation are both treatment options for patients with type 1 diabetes.

“This research is about making stem cells from patients who are alive today. The results provide proof of principle that we can make cell types of interest in a disease—in this case, type 1 diabetes—from an adult patient’s skin sample for potential use in therapeutic cell replacement therapies,” he told *SciBX*.

Egli said that his group is now running studies to compare genetically identical pluripotent human stem cells generated via SCNT and iPS cell reprogramming to understand differences between the two types of cells. His group also is investigating strategies to improve the transplantation of islet β cells and block the autoimmune response against pancreatic cells.

NYSCF has filed a patent application covering the work described in *Nature*. The licensing status is undisclosed.

Public-private partnership roundup

April was a relatively quiet month for public-private partnerships, with the bulk of the activity occurring outside the U.S. Two new consortia—PDE4NPD and SYMPATH—received a total of about €13.8 million (\$19 million) from the EU’s Seventh Framework Programme. Stateside,

The Scripps Research Institute launched Scripps Advance LLC to house assets and IP related to discrete early stage research projects and attract pharma investment in translational assets.⁵ Scripps Advance’s first pharma partner is **Johnson & Johnson** (see Table 1, “Selected public-private partnerships for April 2014”).

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COMPANIES AND INSTITUTIONS MENTIONED

Berg Pharma LLC, Farmingham, Mass.

Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.

Medical University of South Carolina, Charleston, S.C.

The New York Stem Cell Foundation, New York, N.Y.

Scripps Advance LLC, Jupiter, Fla.

The Scripps Research Institute, La Jolla, Calif.

University of Miami Miller School of Medicine, Miami, Fla.