

# Translational tidbits

By Michael J. Haas, Associate Editor, Stephen Parmley and Kai-Jye Lou, Senior Writers, and Lauren Martz, Staff Writer

## Big drive for big data

In the last month, the NIH has pledged almost \$100 million for big data programs aimed at making information usable and accessible to the wider research community and to create a first-of-its-kind database of human cellular responses. The next hurdle will be training and convincing researchers to use the resource.

The Big Data to Knowledge (BD2K) initiative that was launched last December will receive an initial investment of almost \$32 million in 2014 to set up 12 centers, each tasked with solving different challenges related to data collection, storage, analysis, interpretation and sharing. The initiative is expected to receive funding of up to \$656 million by 2020.

The goal is to make it easier for scientists to search across and integrate the large amount of data created by the research community. The money will support development of new software, tools and training programs to help scientists capitalize on results of imaging, genomic, epidemiological and other kinds of studies that generate large amounts of biomedical information.

In addition, the NIH has committed over \$64 million to create a database of human cellular responses to drugs and genetic factors. The funding follows a three-year pilot phase and will go to six research labs at five different institutions—collectively called the Data and Signature Generating Centers.

The selected projects cover a range of cell types and readouts that include protein levels, RNA measurements, mutation status and phosphorylation states, and the Data and Signature Generating Center will maintain a public database in the Library of Integrated Network-based Cellular Signatures (LINCS) system. The **National Human Genome Research Institute** and the **National Heart, Lung, and Blood Institute** will administer the program.

“The underlying premise of LINCS is that disrupting any one of the many steps of a given biological process will cause a ripple of related changes in the molecular and cellular characteristics, behavior or function of the cell—the cellular phenotype—which can be characterized in a variety of cellular assays,” said Ajay Pillai, a program director in the National Human Genome Research Institute’s Division of Genome Sciences and cochief of the LINCS program.

Although investigators at the LINCS data centers will generate signatures from their own data, the hope is that other researchers will help create improved signatures over time. The program directors envision a LINCS knowledge base that would be used by researchers from multiple disease areas.

The LINCS program will also receive support from the BD2K initiative.

## Side by side in Cambridge

In typical academic-industry partnerships, researchers from each organization work on different parts of a project at separate locations, allowing for few real-time, face-to-face interactions between collaborators. **Cancer Research UK** (CRUK) and the **MedImmune LLC** unit of **AstraZeneca plc** are testing a new partnership model that will have pharma and institute scientists working side by side on joint projects in a new facility—the CRUK-MEDI Alliance Laboratory in Cambridge, U.K.

Under the terms of the five-year deal, the partners will focus on early stage R&D of new biologic therapies for cancer. The deal adds to a number of existing partnerships between CRUK and AstraZeneca or MedImmune that span projects in early discovery to advance clinical trials.

CRUK will provide the facilities and equipment along with a portfolio of drug targets and a scientific team. MedImmune will contribute its phage display technology and development expertise, as well as two full-time employees: a scientist with extensive experience with MedImmune’s antibody technologies and drug discovery who will become the new head of the lab, and a bench scientist to provide training and additional expertise to the team.

According to CRUK spokesperson Emma Rigby, the MedImmune employees will spend 80% of their time at the new facility, working alongside a team of institute scientists.

Keith Blundy, CEO of CRUK’s commercial arm, **Cancer Research Technology Ltd.**, told *SciBX*, “We wanted to access world-leading antibody platforms to generate antibody tool compounds and antibody therapeutics.”

He said that the organization spoke to several potential partners but settled on MedImmune in part because of its prior collaborations with CRUK on various clinical projects. He added, “AstraZeneca’s move to Cambridge, along with its openness to new ideas and a willingness to share its technologies, made now an opportune time to approach MedImmune.”

In July, AstraZeneca said that it would build a global headquarters and R&D center in Cambridge by 2016 and would continue expanding its presence in the city in the meantime.<sup>1</sup> That expansion includes plans announced in February to locate up to 60 company scientists at **Cancer Research UK Cambridge Institute’s** labs over the next 3 years.

But Blundy pointed out that the AstraZeneca deal with CRUK is entirely separate from the new MedImmune-CRUK partnership. “As far as I know, AstraZeneca and CRUK have not announced any details about their collaboration,” he said. “Scientists from each organization will be housed in the same building but—as I understand it—each side will be doing its own thing and hoping that something good will come from having the two groups working in proximity.”

Blundy expects the direct collaboration between MedImmune and institute researchers to have project-specific benefits. “The hardest part of working on novel disease biology and drug targets is knowing

**Table 1. Selected public-private partnerships for September 2014.** It was another quiet month for public-private partnerships. The NIH announced a series of 6-year awards totaling about \$64 million to research labs at 5 institutions to create the Library of Integrated Network-based Cellular Signatures, a database of human cellular responses. Other notable activity includes the **MedImmune LLC** unit of **AstraZeneca plc** (LSE:AZN; NYSE:AZN) partnering with **Cancer Research UK** to establish the CRUK-MEDI Alliance Laboratory in Cambridge, U.K., to discover and develop preclinical biologics to treat cancer.

Source: *BioCentury Archives*

Companies	Institutions	Business area	Disclosed value	Purpose
None	<b>Broad Institute of MIT and Harvard; Harvard Medical School; Icahn School of Medicine at Mount Sinai; National Heart, Lung, and Blood Institute; National Human Genome Research Institute; NIH; Oregon Health &amp; Science University; University of California, Irvine</b>	Bioinformatics; Computational chemistry/biology	\$64 million	Partnership to create the Library of Integrated Network-based Cellular Signatures, a database of cellular responses for multiple readouts from a range of cell types
<b>Merck KGaA</b> (Xetra:MRK)	<b>Accelerated Cure Project for Multiple Sclerosis</b>	Autoimmune disease	At least \$1 million	Partnership to carry out a longitudinal clinical study to optimize treatment and understand progression of multiple sclerosis (MS)
MedImmune unit of AstraZeneca	Cancer Research UK	Cancer	Undisclosed	Partnership to establish the CRUK-MEDI Alliance Laboratory to discover and develop preclinical biologics to treat cancer
<b>Johnson &amp; Johnson</b> (NYSE:JNJ)	<b>University of Toronto</b>	Neurology	Undisclosed	Partnership to develop therapies to treat mood disorders and Alzheimer's disease (AD)
Merck KGaA	<b>Massachusetts General Hospital</b>	Autoimmune disease	Undisclosed	Partnership to study systemic lupus erythematosus (SLE) and lupus nephritis
<b>XenoPort Inc.</b> (NASDAQ:XPNT)	NIH	Neurology	Unavailable	Partnership to conduct a clinical trial to evaluate Horizant gabapentin enacarbil to treat alcohol use disorder

robustly that your target really does have the desired effect when modulated in the disease," he said. He anticipates that MedImmune's biological experts will help validate targets early in the discovery process and thus reduce the number of failures caused by pursuing wrong hypotheses.

In turn, Blundy said, "that should enable better definitions of the disease and patient populations, better clinical positioning of the projects and perhaps even smaller trials that can be done more quickly—all of which should lead to greater success rates."

The partners have not yet chosen projects for the collaboration or disclosed financial terms.

### Murdock endowment

David Murdock, owner and chairman of **Dole Food Company Inc.**, announced last month that he will provide a \$15 million annual endowment in perpetuity to the **David H. Murdock Research Institute** (DHMRI) for operating expenses and to attract scientific leadership to the institute.

DHMRI is a not-for-profit CRO on the **North Carolina Research Campus** (NCRC) that was established to offer laboratory technologies to NCRC partners and corporate, academic, government and not-for-profit collaborators.

NCRC was founded by Murdock in 2005 and is a part of **The University of North Carolina** system. NCRC is a public-private partnership made up of 20 industry, technology, healthcare and UNC academic institutions. DHMRI is the first institute on the campus, and its role will be to offer research support to the partners.

DHMRI provides CRO services that include *in vitro* and *in vivo* biomarker discovery and validation, immune monitoring and analysis,

and clinical trial support. The institute is working on indications including diabetes, obesity, rheumatoid arthritis (RA) and cancer. Its goals include promoting nutrition and agriculture science in addition to human health.

The annual \$15 million will go toward attracting researchers to the institute and advancing programs in the human health, nutrition and agriculture research spaces.

The endowment follows a \$50 million pledge to the institute by Murdock last year. That money went toward updating DHMRI's six laboratories and supporting independent research programs in diabetes, metabolomics and cardiovascular biology. Murdock has personally invested over \$800 million in the NCRC as a whole.

### Public-private partnership roundup

Public-private partnership activity in September remained light following a quiet August (see **Table 1**, "Selected public-private partnerships for September 2014").

Notably, **Merck KGaA's** EMD Serono unit and the **Accelerated Cure Project for Multiple Sclerosis** launched a collaborative research program in September to obtain real-world data from patients with multiple sclerosis (MS) that could help optimize treatment and understand disease progression.

EMD Serono will provide at least \$1 million in funding to the Accelerated Cure Project to support the Project's Optimizing Treatment–Understanding Progression (OPT-UP) study. The longitudinal U.S. study is expected to start in 1Q15 and will enroll 2,500 patients with MS and follow them for up to 5 years.

The study aims to both identify factors affecting outcomes in MS to guide treatment decisions and generate data and tools that will

help develop new therapies that could slow, arrest or reverse disease progression.

Researchers will compare the effectiveness, side effects and safety of different MS drugs by collecting and analyzing biological samples, imaging data and treatment outcome data. The researchers also will look for biomarkers associated with mechanisms of progressive MS in addition to predictors and early indicators of response to drugs. Study data and samples will be made available in an open-access forum.

EMD Serono is the biopharmaceutical division of Merck KGaA, which markets the MS drug Rebif interferon beta-1a. The pharma has two other MS compounds in the clinic. Plovamer acetate (PI-2301), a four-amino-acid random sequence peptide copolymer, is in Phase II testing. ATX-MS-1467, in co-development with **Apitope International N.V.**, is a vaccine containing four synthetic peptides derived from human myelin basic protein (MBP) that is in Phase I testing. Finally, the pharma also has an exclusive option to license worldwide rights to develop and commercialize **Opexa Therapeutics Inc.**'s Tcelna imilecleucel-T. The therapy is in Phase IIb testing and consists of patient-specific myelin-reactive T cells (MRTCs) primed and expanded *ex vivo* with myelin-associated peptides.

The partnership with Accelerated Cure Project was EMD Serono's second public-private partnership in September covering autoimmune diseases. Earlier in the month, the company announced a partnership

with **Massachusetts General Hospital** to study systemic lupus erythematosus (SLE) and lupus nephritis.<sup>2</sup>

Haas, M.J. *et al. SciBX* 7(40); doi:10.1038/scibx.2014.1168

Published online Oct. 16, 2014

#### REFERENCES

1. Haas, M.J. *SciBX* 7(32); doi:10.1038/scibx.2014.942
2. Taroncher-Oldenburg, G. *et al. SciBX* 7(36); doi:10.1038/scibx.2014.1061

#### COMPANIES AND INSTITUTIONS MENTIONED

**Accelerated Cure Project for Multiple Sclerosis**, Waltham, Mass.

**Apitope International N.V.**, Diepenbeek, Belgium

**AstraZeneca plc** (LSE:AZN; NYSE:AZN), London, U.K.

**Cancer Research Technology Ltd.**, London, U.K.

**Cancer Research UK**, London, U.K.

**Cancer Research UK Cambridge Institute**, Cambridge, U.K.

**David H. Murdock Research Institute**, Kannapolis, N.C.

**Dole Food Company Inc.** (NYSE:DOLE), Thousand Oaks, Calif.

**Massachusetts General Hospital**, Boston, Mass.

**MedImmune LLC**, Gaithersburg, Md.

**Merck KGaA** (Xetra:MRK), Darmstadt, Germany

**National Heart, Lung, and Blood Institute**, Bethesda, Md.

**National Human Genome Research Institute**, Bethesda, Md.

**National Institutes of Health**, Bethesda, Md.

**North Carolina Research Campus**, Kannapolis, N.C.

**Opexa Therapeutics Inc.** (NASDAQ:OPXA), The Woodlands, Texas

**The University of North Carolina**, Chapel Hill, N.C.