

# Translational tidbits

By Kai-Jye Lou, Senior Writer

At least \$440 million in new support for public-private partnerships was allocated last month globally. Almost \$230 million of that amount is earmarked for the NIH's Accelerating Medicines Partnership<sup>1</sup> (see Table 1, "Selected public-private partnerships for February 2014").

Accelerating Medicines Partnership members will collaborate on projects to identify and validate disease targets in Alzheimer's disease (AD), type 2 diabetes, rheumatoid arthritis (RA) and systemic lupus erythematosus (SLE). Over the public-private partnership's 5-year run, the NIH will provide \$118.9 million in funding, and industry partners will provide the remaining \$110.6 million in the form of funding and in-kind contributions.

The second-largest public-private partnership announced in February was the European Gram-Negative Antibacterial Engine (ENABLE) project, which is getting \$116 million over 6 years and is focused on developing antibacterial compounds. In third place was the ARTERIA project, which has received C\$49.2 million (\$44.6 million) and is focused on cardiovascular disease.

### Antibacterial engine

**Table 1. Selected public-private partnerships for February 2014.** Over \$440 million in new funding commitments and in-kind contributions was earmarked for public-private partnerships in February. This was primarily driven by the NIH's Accelerating Medicines Partnership, the Innovative Medicines Initiative (IMI)'s European Gram-Negative Antibacterial Engine (ENABLE) project and the Montreal Heart Institute's ARTERIA project.

Source: BioCentury Archives

Companies	Institutions	Business area	Disclosed value	Purpose
AbbVie Inc. (NYSE:ABBV); Biogen Idec Inc. (NASDAQ:BIIB); Bristol-Myers Squibb Co. (NYSE:BMJ); Eli Lilly and Co. (NYSE:LLY); GlaxoSmithKline plc (LSE:GSK; NYSE:GSK); Johnson & Johnson (NYSE:JNJ); Merck & Co. Inc. (NYSE:MRK); Pfizer Inc. (NYSE:PFE); Sanofi (Euronext:SAN; NYSE:SNY); Takeda Pharmaceutical Co. Ltd. (Tokyo:4502)	NIH	Autoimmune disease; endocrine/metabolic disease; neurology	\$229.5 million	Accelerating Medicines Partnership to identify and validate disease targets, with an initial focus on Alzheimer's disease (AD), type 2 diabetes, rheumatoid arthritis (RA) and systemic lupus erythematosus (SLE)
GlaxoSmithKline	IMI; Uppsala University	Infectious disease	€85 million (\$116 million)	ENABLE project to progress antibacterial research programs through discovery and Phase I testing
Roche (SIX:ROG; OTCQX:RHHBY); Servier; AstraZeneca plc (LSE:AZN; NYSE:AZN); Valeant Pharmaceuticals International Inc. (TSX:VRX; NYSE:VRX)	Montreal Heart Institute	Cardiovascular disease; diagnostics; genomics	C\$49.2 million (\$44.6 million)	ARTERIA project to develop treatments and diagnostics for cardiovascular disease

(Continues on p. 2)

Last month, the **Innovative Medicines Initiative** (IMI) launched the third project under its New Drugs 4 Bad Bugs (ND4BB) initiative. The ENABLE project aims to build and manage a discovery platform for testing and optimizing early discovery-stage molecules for drug-resistant, Gram-negative bacterial infections.

The seven candidates already in the project's portfolio will be reviewed by ENABLE's Portfolio Management Committee before entering the development pipeline. One candidate each was sourced from **Redx Pharma Ltd.**, **Northern Antibiotics Ltd.** and **biomol-informatics S.L.**; the other four came from the not-for-profit research organization **Medina Foundation** and from a trio of European universities.

"In theory, a maximum of four candidates can be developed at any one time, and more candidates will be sought to feed in as candidate programs are stopped and capacity becomes available," said ENABLE spokesperson Claire Skentelbery, who is secretary general at biotech trade organization **European Biotechnology Network**. "We will launch an open call at the start of March to begin the process of identifying new candidates external to the current consortium that can feed into the pipeline as space becomes free."

The IMI will provide ENABLE with €59 million (\$80.5 million) in funding, and project members will provide another €26 million (\$35.5 million) in in-kind contributions. **GlaxoSmithKline plc** and **Uppsala University** are leading the project.

The project's goal is to complete Phase I trials for at least one candidate by 2019. ENABLE currently has 32 members, including 3 pharmas and 11 small and medium enterprises.

The other two ongoing projects launched under IMI's ND4BB initiative are COMBACTE and TRANSLOCATION. COMBACTE is

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— Claire Skentelbery,  
European Biotechnology Network

**Table 1. Selected public-private partnerships for February 2014.** (Continued)

Companies	Institutions	Business area	Disclosed value	Purpose
European Federation of Pharmaceutical Industries and Associations	Pfizer–University of Granada–Junta de Andalucía Centre for Genomics and Oncological Research (GENYO); IMI	Autoimmune disease	€22.7 million (\$31.1 million)	Five-year PRECISESADS research project to provide a molecular map to guide therapy in systemic autoimmune diseases
ImaginAb Inc.	Duke–NUS Graduate Medical School; National Research Foundation	Cardiovascular disease; cancer; endocrine/metabolic disease	\$15 million	Partnership to establish Imaging Biomarker Development Lab to develop <i>in vivo</i> molecular imaging agents for cardiovascular and metabolic disease and cancer
California Stem Cell Inc.	California Institute for Regenerative Medicine; University of California, Irvine	Ophthalmic disease	\$4.5 million	Partnership to develop human stem cell-derived, transplantable, 3D retinal tissue to treat incurable retinal diseases, such as retinitis pigmentosa and age-related macular degeneration (AMD)
Fluidda N.V.; Materialise N.V.	University of Antwerp; Columbia University; University of Pennsylvania	Pulmonary disease; transplantation	€1 million (\$1.4 million)	Consortium to detect signs of rejection after lung transplantation using Fluidda's functional respiratory imaging
Abivax S.A.S.	Cuban Center for Genetic Engineering and Biotechnology	Infectious disease	Undisclosed	Partnership to co-develop a therapeutic vaccine in Phase IIB testing to treat chronic HBV infection
Audentes Therapeutics Inc.	Genethon	Gene/cell therapy; musculoskeletal disease	Undisclosed	Partnership to develop Audentes' AT001 to treat X-linked myotubular myopathy
AstraZeneca	University of California, San Francisco	Pharmaceuticals	Undisclosed	Three-year partnership to discover and develop small molecules and biologics to treat a range of indications
DBV Technologies (Euronext:DBV)	Icahn School of Medicine at Mount Sinai	Autoimmune disease	Undisclosed	Partnership to conduct preclinical testing of epicutaneous antigens via DBV's Viaskin skin patch technology to treat Crohn's disease
GlaxoSmithKline	The University of Edinburgh	Hepatic disease	Undisclosed	Partnership under GSK's Discovery Partnerships with Academia to discover and develop treatments for liver fibrosis or cirrhosis
RaQualia Pharma Inc. (JASDAQ:4579)	Nagoya University	Neurology; gastrointestinal disease	Unavailable	Partnership to identify a drug candidate, with a focus on pain and GI indications
Pfizer	Massachusetts Institute of Technology	Pharmaceuticals	Undisclosed	Three-year partnership to translate discoveries in synthetic biology to advance drug discovery and development technologies, including cellular genome engineering

focused on developing innovative trial designs for antibacterial agents, whereas TRANSLOCATION is researching the cell permeability of Gram-negative bacteria.

#### A hearty investment

The **Montreal Heart Institute** launched its ARTERIA project to aid the development of new treatments and diagnostics for cardiovascular disease.

The project's goal is to use genetic profiles to improve treatment outcomes in patients who have or are at risk for cardiovascular diseases such as atherosclerosis.

ARTERIA has five focus areas. These include validating diagnostics for statin-induced muscle toxicity and incorporating them into care regimens; running studies to validate genetic markers and other biomarkers to identify patients who respond well to high-density

lipoprotein-targeting therapies; running studies to validate a plasma biomarker to help predict a favorable response to vascular anti-inflammatory agents; determining whether various anti-inflammatory agents produce measurable vascular effects and clinical benefits in vascular disease patients; and identifying genetic factors that could help determine the benefits of drugs designed to lower heart rates.

The government of Quebec has provided ARTERIA with C\$18.2 million (\$16.5 million) in funding, and industry partners have contributed another C\$31 million (\$28.1 million) in the form of private investments. The Montreal Heart Institute is leading the project.

ARTERIA's industry partners include **Roche**, the Servier Canada Inc. subsidiary of **Servier**, the **MedImmune LLC** unit of **AstraZeneca plc** and the Valeant Canada Inc. subsidiary of **Valeant Pharmaceuticals International Inc.**

### Grant news is good news

February saw the **Cancer Prevention & Research Institute of Texas** (CPRIT) announce its first round of grants since December 2012.

CPRIT distributes taxpayer money to Texas-based academic and industry cancer researchers. The institute ran into problems in mid-2012 when then-CSO Alfred Gilman accused the institute's executive leadership of tampering with the scientific peer review of grant proposals.<sup>2</sup>

Texas Gov. Rick Perry and state legislators froze CPRIT's grant-giving authority in December 2012 and asked the agency to create a new plan for vetting grant proposals. The state lifted the moratorium last October, and the agency issued a new call for proposals two months later.<sup>3</sup>

In its new round of funding, CPRIT awarded 3-year grants totaling \$63.2 million to 6 cancer companies that are based in or planning to relocate to Texas. The agency also awarded 4 research and 7 training grants totaling \$22.9 million to academic and medical centers across the state.

Among companies relocating to the state, Maryland's **Beta Cat Pharmaceuticals Inc.** will receive up to \$15.9 million to develop inhibitors against an undisclosed cancer target in the  $\beta$ -catenin (CTNNB1) pathway.

Michigan's **ProNAi Therapeutics Inc.** will receive up to \$14 million for Phase II testing of PNT2258, a cancer therapeutic targeting a DNA sequence upstream of B cell lymphoma 2 (BCL-2; BCL2) promoters.

Canada's **Essa Pharma Inc.** will receive up to \$12 million for a program to develop a blocker of the N-terminal domain of the androgen receptor for prostate cancer.

The three in-state companies getting CPRIT grants are **DNatrix Inc.**, **CerRx Inc.** and **ProPep Surgical LLC.**

DNatrix will get up to \$10.8 million to develop a genetically modified adenovirus to treat glioblastoma. The company's DNX-2401 is in Phase I testing for recurrent glioblastoma multiforme (GBM). CerRx is getting up to \$6 million to develop ceramide-modulating therapeutics to treat cancer. The company's lead compound is an i.v. formulation

of the synthetic retinoid derivative fenretinide that has completed Phase I trials.

Fenretinide is known to decrease ceramide synthesis.

ProPep will get up to \$4.4 million to do real-time nerve identification during robotic prostatectomy, which could help decrease post-surgery rates of sexual dysfunction and urinary incontinence.

Recipients of CPRIT research grants are the **Baylor College of Medicine**, **Rice University**, **The University of Texas Medical Branch** and **The University of Texas Southwestern Medical Center.**

Rice will get up to \$3.9 million for a project to identify new biomarker signatures of cancer and its progression and to develop new diagnostic and screening tests for such cancers. UT Medical Branch will get up to \$3.2 million to perform comparative effectiveness research on the care and treatments provided to patients with cancer in Texas. Baylor will get up to \$2.2 million to study genetic events that lead to metastasis of osteosarcoma and to develop targeted therapies for the disease. UT Southwestern will get up to \$1.8 million to develop new techniques to image the size and metabolic state of a tumor.

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3. Osheroich, L. *SciBX* 5(43); doi:10.1038/scibx.2012.1128

### COMPANIES AND INSTITUTIONS MENTIONED

**AstraZeneca plc** (LSE:AZN; NYSE:AZN), London, U.K.  
**Baylor College of Medicine**, Houston, Texas  
**Beta Cat Pharmaceuticals Inc.**, Gaithersburg, Md.  
**biomol-informatics S.L.**, Madrid, Spain  
**Cancer Prevention & Research Institute of Texas**, Austin, Texas  
**CerRx Inc.**, Lubbock, Texas  
**DNatrix Inc.**, Houston, Texas  
**Essa Pharma Inc.**, Vancouver, British Columbia, Canada  
**European Biotechnology Network**, Brussels, Belgium  
**GlaxoSmithKline plc** (LSE:GSK; NYSE:GSK), London, U.K.  
**Innovative Medicines Initiative**, Brussels, Belgium  
**MedImmune LLC**, Gaithersburg, Md.  
**Medina Foundation**, Granada, Spain  
**Montreal Heart Institute**, Montreal, Quebec, Canada  
**National Institutes of Health**, Bethesda, Md.  
**Northern Antibiotics Ltd.**, Helsinki, Finland  
**ProNAi Therapeutics Inc.**, Kalamazoo, Mich.  
**ProPep Surgical LLC**, Austin, Texas  
**Redx Pharma Ltd.**, Liverpool, U.K.  
**Rice University**, Houston, Texas  
**Roche** (SIX:ROG; OTCQX:RHHBY), Basel, Switzerland  
**Servier**, Neuilly-sur-Seine, France  
**The University of Texas Medical Branch**, Galveston, Texas  
**The University of Texas Southwestern Medical Center**, Dallas, Texas  
**Uppsala University**, Uppsala, Sweden  
**Valeant Pharmaceuticals International Inc.** (TSX:VRX; NYSE:VRX), Montreal, Quebec, Canada