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POTENTIAL BIGGEST EVER UK BIOTECH IPO

Malin eyes $360M Dublin IPO, as Cardio3, Quantum Genomics complete new share issues

By Cormac Sheridan, Staff Writer

DUBLIN – Funding continues to flow to European biotech, with Cardio3 Biosciences SA and Quantum Genomics SA raising €32 million (US$36 million) and €12.9 million (US$14.44 million), respectively, in secondary offerings last week. Both transactions confirm the current appetite among investors for European biotech, but a much sterner test of that support is on the near-term horizon, as a new Irish-American firm Malin Corp. plc unveiled ambitious plans to raise €275 million to €325 million in an IPO in Dublin shortly.

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YOUR BEST DEAL

Nominees announced for Allicense Breakthrough Deal Awards

By Peter Winter, BioWorld Insight Editor

Thomson Reuters analysts have disclosed 10 deals – five biopharma licensing and five M&A deals concluded in 2014 – as candidates for the Breakthrough Alliance Awards, with voting now open and the winner to be

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IN THE CLINIC

AHRQ's RoPR off & running, but building awareness is critical

By Marie Powers, Staff Writer

In 2012, the Agency for Healthcare Research and Quality (AHRQ) set out to develop the Registry of Patient Registries (RoPR) as a one-stop repository of information about patient registries

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THE BIOWORLD BIOME

CROI 2015

HIV not the only important virus at CROI conference

By Anette Breindl, Science Editor

Researchers gathered in Seattle last week to share their latest progress in fighting HIV and the infectious diseases that come in its wake at the 2015 annual

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FINANCINGS

Chiasma prepping solo U.S. oral octreotide plan with $70M series E

By Jennifer Boggs, Managing Editor

Rather than seeking a new partner after a worldwide licensing deal with Roche AG quietly terminated last year, privately held Chiasma Inc. decided to take its new drug application (NDA)-ready

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IN THIS ISSUE

BENCH PRESS

BioWorld Science Editor Anette Breindl takes a closer look at **translational medicine**

Read this week's edition

IN THE CLINIC

BUT THERE'S BACKUP

Skin crawl: BI BACE pace slowed by phase I effect; Vitae drug held in clinic

By Randy Osborne, Staff Writer

Boehringer Ingelheim (BI) GmbH's clinical hold on the oral beta secretase (BACE) inhibitor BI 1181181 for Alzheimer's disease (AD) because of skin reactions in some

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focused on, but not exclusive to, U.S.-based registries. The goals, in a nutshell, were to increase collaboration, reduce redundancy and improve transparency during drug and medical device development. (See *BioWorld Today*, July 13, 2012.)

The mechanism to achieve that end was the creation of a searchable database compatible with ClinicalTrials.gov that facilitates the use of common data fields and definitions across health conditions, improving opportunities to share, compare and link data. RoPR also is designed to serve as a public repository of searchable summary results and as a recruitment tool for researchers and patients seeking to participate in patient registries.

After three painstaking years, RoPR now encompasses 100 patient registries. But that's a drop in the bucket, AHRQ officials conceded. A re-energized effort now is seeking to expand the size and value of RoPR to the research community.

RoPR was designed to function for patient registries much the same way that ClinicalTrials.gov does for clinical trials. Many registries can be used for a variety of purposes, including effectiveness research for drugs or devices and studies of the natural history of a disease, postmarketing commitments or safety monitoring and quality improvement programs. AHRQ's intention was to make RoPR a natural extension of the clinical research process, enabling researchers to connect with patients and access existing data for use in new studies so they could enroll and complete trials faster and at a lower cost.

In fact, access to list a patient registry profile on RoPR is granted by posting a record in ClinicalTrials.gov's protocol registration system (PRS) (https://register.clinicaltrials.gov/). Once that record is created and identified as a patient registry, a link is created in PRS to the RoPR registration system, allowing users to list a registry profile.

Since patient registries often serve as a type of observational study, sponsors are not required to post them on ClinicalTrials.gov, but many continue to do so. However, users often fail to take the extra step of completing a registry on RoPR.

The first order of business in expanding RoPR is to close that loop, said Michelle Leavy, manager for health policy in the real world late phase research program at Quintiles Inc., which worked with AHRQ to spearhead the design and development of RoPR.

"To date we've seen good participation from the industry side," with many companies providing valuable feedback on what type of information to capture in RoPR, Leavy told *BioWorld Today*. "We were working hard to collect the most relevant information but also a minimum amount of information so we weren't overburdening people."

RoPR also has attracted "a diverse group of registries," Leavy said, both in terms of disease types and geographic mix.

And RoPR is beginning to foster the types of collaborations for which it was designed. Leavy cited the CERTAIN sub-study of the CORRONA registry – an ongoing longitudinal rheumatoid arthritis (RA) registry that was established in 2001 – that is being conducted by the Consortium of Rheumatology Researchers of North America in conjunction with Genentech Inc. CERTAIN is using the CORRONA registry to conduct a comparative effectiveness study of biologics used to manage RA, including Enbrel (etanercept, Amgen Inc.), Humira (adalimumab, Abbvie Inc.), Remicade (infliximab, Johnson & Johnson), Cimzia (certolizumab pegol, UCB SA), Simponi (golimumab, Janssen Biotech) and others.

"Instead of going off and developing a new registry, Genentech's been able to partner with this existing group, nest its study within the registry and conduct it in an efficient way," Leavy explained. "That's the type of collaboration we want to encourage."

MANY BARRIERS TO COLLABORATION

But registration on RoPR is voluntary, and Leavy estimated that only 10 percent to 15 percent of the observational studies registered in ClinicalTrials.gov have taken the extra step, which requires the completion of approximately 40 additional questions.

"We need to have a critical mass of registries," acknowledged Elise Berliner, director of technology assessment programs in AHRQ's Center for Evidence and Practice Improvement.

Key to recruiting more registries is helping users to understand the difference between RoPR and ClinicalTrials.gov. Questions used in the two systems are not redundant. Those specific to RoPR are geared more to the characteristics and use of patient registries, enabling researchers to look at topics such as the natural history of disease.

"RoPR is a much different undertaking than ClinicalTrials.gov," where information about ongoing and completed trials are shared primarily by investigators and sponsors for use by other researchers as well as clinicians, patients and the public, observed John Lewis, senior vice president for policy and public affairs at the Association of Clinical Research Organizations (ACRO).

"With registries, you're looking at people who have certain conditions who are largely volunteering information to feed into that research," he said. "We're trying to find and encourage ways where the research can actually inform the clinical trial process for research into new and better treatments. That's the reason a lot of these registries were created."

Although AHRQ is well-positioned to house RoPR, lack of awareness has hindered uptake, he suggested.

"There is a fair amount of awareness around ClinicalTrials.gov," he pointed out. "There's not been much promotion or dissemination of the availability of the Registry of Patient Registries."

Many ACRO members set up and run registries for patient

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Malin

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The precise timing depends, however, on the outcome of discussions with the FDA about whether it can use its proprietary C-Cathez catheter in the study.

"We believe it would be beneficial to use our own catheter in the trial," Homsy said. "To use it in an IND trial, you need specific authorization." When – or if – that will come is unclear as yet, even though the same device already has European approval.

"The FDA has its own timelines," Homsy said. "This is really not in our hands, unfortunately."

A phase III trial is ongoing in Europe. A final efficacy readout is due in the second quarter of 2016, but the outcome of an interim futility analysis is expected about a month from now.

Quantum Genomics priced its offering of 2,049,875 new shares at €6.30 per share, at the top of the indicative price range of €5.40 to €6.30 it had set at the outset of the fundraising process.

The total raised included an overallotment option of 267,375 shares, which the lead manager and bookrunner Invest Securities exercised in full. The company aims to use the proceeds to fund a phase IIa trial of its first-in-class anti-hypertensive QGC001, a brain aminopeptidase inhibitor. (See *BioWorld Today*, Feb. 4, 2015.) //

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groups, according to Lewis. Having the ability to identify new uses for patient-reported information could represent a huge benefit to industry in designing new trials and targeting certain populations – especially to the extent that the data are aggregated across large populations.

Quintiles is encouraging its contacts in pharmaceutical and medical device industries to post their studies on the RoPR. The company also is working with AHRQ to improve RoPR's educational content, designed to help users learn about best practices in building patient registries. Leavy was among the team that assisted AHRQ in developing a guidebook, Registries for Evaluating Patient Outcomes, that is cited in guidance documents from the Centers for Medicare & Medicaid Services and from the FDA.

A second priority is to familiarize more patient groups with RoPR. Last month, AHRQ worked with a number of organizations, including the American Medical Association's National Quality Registries Network, to host a "registration-a-thon" seeking to grow the number of registries in RoPR.

"The stakeholder community has been very enthusiastic about RoPR," Berliner said.

In addition, AHRQ recently funded a grant to find patient-generated registries, such as Patients Like Me, and help them connect.

Although the process of informed consent can be a concern with respect to accessing data from patient registries, RoPR collects only aggregated data about each registry rather than actual patient information.

RoPR also offers the opportunity for researchers to evaluate data using common data elements, Lewis said, ultimately setting up the opportunity for interoperability among registry data, clinical care data and clinical trials data to help researchers get a fuller picture of patients.

"If you can tie the clinical outcomes to the patient-reported outcomes and the clinical trial results, you can have better targeted trials and better patient recruitment while minimizing adverse events," Lewis said. That's the longer term vision."

Ultimately, the full impact of RoPR will be realized through collaborations between patient registries in RoPR, Berliner said.

"There are many barriers to this type of collaboration, including issues of patient privacy, governance and ownership of data," she told *BioWorld Today*, noting that such issues are addressed in the handbook.

The RoPR project also is working to set up a web-based user forum to facilitate additional discussions among registry holders and other stakeholders. "We are working on the design of the web-based forum now," Berliner said.

"In order to be able to compare outcomes across different registries, we need to understand whether the different registries actually measure the same thing," she added. "We are working on a review of systems other groups have developed to harmonize outcome measures, and this review will be posted soon for public comment. In the next few years, we hope to incorporate an outcome measures framework into RoPR so that users will be able to easily compare definitions across registries." //

Allicense

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Sanofi will lead global development and commercial activities for DCM, where it obtained worldwide rights, and ex-U.S. regulatory and commercial activities to the two HCM programs, where it holds ex-U.S. rights. (See *BioWorld Today*, Sept. 18, 2014.)

CAST YOUR VOTE

The voting process on these deals is now open and you can record your selection for a deal in both the M&A and License category at https://www.surveymonkey.com/s/5SS5W9Y.

The winners will be announced at the upcoming Allicense 2015 conference that will be held May 5-6, 2015, in San Francisco (www.allicense.com).

The meeting will feature keynote speaker Billy Beane, the legendary general manager of the Oakland A's baseball team, who will share his thoughts on applying Moneyball – the concept that he successfully deployed in baseball – to biotech. //