



NovaSecta Quarterly

17 October 2011

Welcome to the NovaSecta Quarterly for Q3 2011, in which we share our perspectives on selected industry news and developments in the last quarter, viewed in relevance to Pharma/Biotech organisations of small to mid-sized scale, 'MidPharmas'.

Companies featured this quarter:

- **Corporate Development:** AstraZeneca, Biotie, BTG, Evotec, Exonhit, Galapagos, GSK, Hikma, Innate, Lundbeck, Newron, Photocure, Platine, Proximagen, Roche, Transgene, UCB, Vectura, Wellstat Therapeutics
- **Research & Development:** Antisense, Basilea, Crucell, Cubist, Durata, Humabs Biomed, Institute for Research in Biomedicine, Optimer, Oxford Biomedica, Sanofi, Santaris, Symphogen, Rib-X, Theraclone, Trius, UK Medical Research Council
- **Marketing & Sales:** BioAlliance, Biogen Idec, C&O, GSK, Orphan Europe, Shanghai Pharma, Shiongi, Shenzhen Neptunus, Sobi

Corporate Development

European MidPharmas highly active in the CNS field

During the last quarter we observed with interest activity in the CNS space from Biotie, Newron, Lundbeck, Evotec, UCB and Exonhit. The Finnish company [Biotie acquired Italian Newron Pharmaceuticals](#), the latter which enjoyed a successful quarter with [US IND approval](#) for NW-3509, an add-on therapy for schizophrenia, and also [completed patient enrolment](#) in a pivotal phase III trial of safinamide for advanced Parkinson's disease. Meanwhile, Lundbeck made a [£10.3m investment in Proximagen Group](#), a British company developing a number of candidates for CNS disorders including [assets acquired from GSK](#). Evotec signed an agreement with Roche for its [MAO-B inhibitor for patients with Alzheimer's disease](#), and a [collaboration with UCB](#) for multi-target drug discovery in CNS disorders. Finally, Exonhit initiated a research collaboration with Pfizer to [identify Alzheimer's disease biomarkers](#). These investments demonstrate European MidPharmas are very much up to the challenge of exploiting the CNS space that some Big Pharmas, including GSK and AstraZeneca, have [de-prioritised](#).

Mixing of own R&D with service business

Combining internal R&D with fee-for-service activities, employed by Galapagos and Evotec amongst others, illustrates a business model that provides additional sources of revenue while optimising FTE utilisation. A variation of this is the collaboration by Transgene and Innate Pharma to perform service work, through a novel joint venture called [Platine Pharma Services](#). Platine, owned equally by the two companies, will offer fee-based immunomonitoring services to the European market. This is an ideal opportunity to leverage internal expertise, both for innovative drug development and the establishment of an independent revenue stream. We expect to see more business model innovation of this kind as Pharma/Biotechs continue to seek non-dilutive revenue sources.

European MidPharmas make further inroads in the USA

The last quarter saw a number of European companies establishing or reinforcing their presence in the US; with licensing agreements, new product launches and initiation of US clinical trials. UK-based BTG acquired the US marketing rights for an [investigational antidote for 5-FU overexposure](#) from Wellstat Therapeutics, leveraging the former's already established sales and support team in the US. Another British company Vectura signed a [US development agreement](#) with an undisclosed leading Pharmaceutical company, securing the future of its investigational drug

VR315 for asthma / COPD. Meanwhile Hikma launched [therapeutic heparin in the US](#), and earlier this year Nordic Photocure launched Allumera, [a photodynamic therapy based cosmetic product](#), only in the US. In addition, Newron received approval to [start its first in-man trial in the US](#) for its candidate add-on therapy for schizophrenia. These activities exemplify various tactics used for establishing an initial foothold or securing and growing presence in the US. They also highlight the continued attraction of the world's biggest drug market for European MidPharmas.

Research & Development

US Biotechs and MidPharmas innovate in antibiotics

Only a few Big Pharmas have active antibiotic R&D programs and indeed, in the last four decades only [two new classes](#) of antibiotics have reached the market. This may be changing however, given the recent R&D activities: the FDA just [approved fidaxomicin](#) from Optimer Pharmaceuticals, the first drug in over two decades for the treatment of *C. diff* infections. Its co-promotion partner, Cubist Pharmaceuticals, [announced](#) the initiation of its own phase III study for the treatment of *C. diff*-associated diarrhoea. In addition, [Trius Therapeutics](#) and [Durata Therapeutics](#) both started independently their second global phase III pivotal trials for treatment of acute bacterial skin and skin structure infections. Rib-X Pharmaceuticals and Basilea were even more ambitious, tackling multi-drug resistant Gram-negative infections including the new NDM-1 strains that inactivated the last safe and effective antibiotics. [Rib-X announced](#) a research collaboration agreement and an option to license with Sanofi for its RX-04 antibiotics program, while [Basilea initiated](#) its second phase I study against a range of multidrug-resistant Gram-negative pathogens, including NDM-1. It is interesting to note that innovation in antibiotics R&D is currently driven by small and mid-sized companies, most of which are based in the US - the Swiss-based Basilea being a notable exception.

Promising platform technologies prove their value

A strong platform technology has provided a solid foundation for many small to mid-sized Biotechs and Speciality Pharma companies. The last quarter saw positive announcements from Antisense, Santaris, Oxford Biomedica and Symphogen, all working with proprietary technology platforms. Antisense Pharma, developing targeted therapies for malignant tumours using its .R.A.D.A.R.[®] technology for the identification of antisense molecules as drug candidates, was typical of the good news: the company [completed a financing round](#) of approximately €8 million,

presented [positive results](#) from its on-going clinical phase I/II trial, and [started preparation for a pivotal phase II/III study](#) on pancreatic cancer aimed for the first second-line therapy. Both studies are based on the drug Trabedersen, a first-in-class gene silencing antisense compound [already in phase III](#) for an especially aggressive form of brain cancer. There was also positive news from Santaris Pharma, developing microRNA and mRNA-targeted therapies using its proprietary Locked Nucleic Acid Drug Platform, for its candidate product [miravirsen and its phase IIa trial](#) for Hepatitis C. Further, Oxford Biomedica, developing gene-based therapies using the company's LentiVector® gene delivery technology reported positive interim data from [ProSavin® phase I/II study in Parkinson's disease](#). Finally Symphogen, developing recombinant antibody mixtures using proprietary antibody discovery platforms announced the initiation of a phase II trial for the investigation of [Sym004 for recurrent and/or metastatic squamous cell carcinoma of the head and neck](#). These announcements further demonstrate how a persistent focus on distinctive capabilities – in this case platform technologies – is proving successful for MidPharmas.

Small to mid-sized companies discover innovative antibodies for the influenza A virus

Each year, vaccines against [selected illness-causing strains](#) of influenza are developed, protecting against influenza B and against 2 of the 16 subtypes of influenza A. The ultimate flu vaccine would target any strain of any subtype of influenza A, thus effectively protecting against seasonal flu and also against pandemic flu outbreaks. Such work is currently underway: Crucell (now a part of J&J but still apparently operating well at a mid-sized scale) recently [identified two antibodies](#), each protecting against one of the two main groups of influenza A, both of which have caused human pandemics. Crucell believes that a combination of these antibodies may be sufficient to develop a vaccine that could protect against most influenza A subtypes. Scientists from [Humabs BioMed \(a spin-out from the Institute for Research in Biomedicine\)](#) and the [UK Medical Research Council](#) have gone one step further: developing the world's first antibody that has proven to target all 16 subtypes of influenza A. On a similar note, VC-backed Theraclone Sciences has [just initiated a phase I clinical trial](#) for an antibody that could shield against a spectrum of influenza A strains. Although it is still early days, these innovative antibodies could one day lead to new "universal" vaccines protecting effectively against seasonal and pandemic flu.

Marketing & Sales

MidPharmas push forward in orphan drug development

The orphan drug sector is increasingly attractive to Pharmas of all sizes, and the last quarter saw several promising clinical results from mid-sized players. In July, Sobi and Biogen Idec published [positive phase I/IIa data for their recombinant factor VIII therapy \(rFVIII-Fc\)](#) for the prophylactic treatment of severe haemophilia A. BioAlliance Pharma's orphan oncology portfolio was strengthened by [Livatag's® application validation](#) for a phase III trial for the treatment of primary liver cancer, and [Lauriad's™ orphan drug designation](#) by EU and FDA for the prevention of radiotherapy-induced oral mucositis in cancer patients. Meanwhile Orphan Europe (a subsidiary of Recordati) recently established a [partnership with E-IMD](#) (European Registry and Network for Intoxication type Metabolic Disease), emphasising the need for more access to research, treatment and patient data usually scarce for orphan diseases. Such advances and initiatives in orphan drug development are particularly encouraging for MidPharmas already active in this space, and provide attractive entry points to various therapeutic markets for those looking to exploit the orphan indication route-to-market and incentives.

Chinese Pharmas seeking US / European acquisitions and partnerships

Shanghai Pharma – China's second largest Pharmaceutical distributor – announced their goal of acquiring a [US or European Pharma within the next year](#), and their target would likely be a mid-sized company owning patented products. The fast-growing size and momentum of emerging markets have rendered Chinese Pharmas attractive acquisition and partnership targets for Western and Japanese players alike. For instance, [GSK offered to purchase the remaining 51% stake of Shenzhen Neptunus](#) and become the sole owner of this previously formed joint venture. Similarly, Japan's Shionogi agreed to buy a [24% stake from China's C&O Pharma](#), gaining access to the company's lucrative products and distribution networks. If successful, Shanghai Pharma's acquisition would not only expand the company's product portfolio and raise its global competitiveness, but also potentially pioneer foreign buyouts and mark a significant milestone in the Chinese Pharma industry as a whole. It will be interesting to note which US or European MidPharma(s) have been shortlisted, as this may indicate the types of products and therapy areas that are attractive to Chinese buyers.

Look out for the next issue of the NovaSecta Quarterly out January 2012

About NovaSecta

NovaSecta is the specialist MidPharma management consulting firm: our focus is small to mid-sized Pharmaceutical and Biotech companies. We work with clients to evolve their capabilities to exploit changing industry realities, create markets and seize new opportunities, and initiate and manage collaborations with diverse external partners. Our client work spans Corporate Development, Research & Development, and Marketing & Sales.

For further details on our work and our team, please visit our [website](#), and see below for our upcoming multi-company events:

R&D Leaders Meeting	Corporate Development Meeting: Pharma Network	NEW! Strategic Marketing Meeting
24th November 2011 Copenhagen	10th May 2012 London	16th February 2012 London

To register your interest in our multi-company events, please [contact us](#).