

News Story October 23, 2001

Waterworth C: **BioPartnering Europe 2001 - Ninth Annual Conference (Part VII), Investing in the Convergence of Biotech and IT, London, UK, 14-16 October 2001, October 14-16 2001 Iddb Meeting Report (23-Oct-2001)**

The young ones

"Out of every five companies set up now, in ten years, one will survive, one will be bought and three will disappear," **estimated William J Kridel Jr, MD of Ferghana Partners (USA)**. BioPartnering Europe (BPE) has traditionally presented an unmissable opportunity for small start-up companies to promote themselves and to catch the eye of attendant venture capitalists and possible business partners. Of the many companies at the meeting, representatives of three spoke to Current Drugs about their company's milestones, research platforms and future prospects.

Achillion Pharmaceuticals

Kevin Eastwood, Director of Business Development, spoke about Achillion Pharmaceuticals Inc (USA), which was established in February 2000. Since that date, \$24 million in private equity have been raised, and the number of employees has risen from four persons to 45. Achillion is partnered with Yale University and is focused on the discovery, development and commercialization of innovative therapies for infective diseases. The company ultimately partnered with Yale to bring compounds from Yung-Chi Cheng's laboratory to the clinic; it is hoped that these compounds will generate near term revenue potential.

Mr Eastwood talked briefly of Achillion's pipeline candidates. The lead compound in the first group of compounds is beta-LFd4C (Achillion Pharmaceuticals Inc/Yale University/VionPharmaceuticals Inc). This agent targets HIV and HBV viruses for the indications of HIV and AIDS infection and chronic hepatitis B, respectively. It is currently in phase Ib clinical trials. The second candidate is a zinc finger inhibitor (Achillion Pharmaceuticals Inc/National Cancer Institute/National Institute of Allergy and Infectious Diseases) which targets the HIV virus and is currently pre-clinical. The final candidate, also pre-clinical, is L-OddU (Achillion Pharmaceuticals Inc), which targets EBV for the potential treatments of post-transplant lymphoproliferative disorder and infectious mononucleosis.

It is anticipated that, within two years, beta-LFd4C will have completed the phase II pivotal study, and so it will become clear whether beta-LFd4C is likely to be a success in the clinic, an outcome which will clearly impact upon the company's balance sheets. The company is also aiming to develop the zinc finger technology for indications besides HIV, and hopes to have a compound that is close to NDA filing.

Mr Eastwood maintained that the current climate is not hostile to start-up companies. Achillion have endeavored to communicate with investors and to explain the significance of the companies investments and which milestones will be achieved. He revealed that Achillion would soon be closing their next financial round and that it would be up on the

previous round.

BASILEA Pharmaceutica

Tony Mann, CCO, and Mr Ron Scott, CFO, spoke in depth about their company, BASILEA Pharmaceutica (Switzerland), beginning by giving some background on the company.

The focus of Basilea is the discovery and development of novel drugs for the treatment of bacterial infections, fungal infections and skin diseases. The company was founded in 2000 as a spin off of F Hoffmann-La Roche Ltd; it was borne of a desire by Roche to concentrate on its anti-infective agents and to formulate a blockbuster strategy in this arena. Roche still has opt-in rights at market rates on seven defined molecules at the end of phase II development and benefits from its retained 49% equity interest; Basilea remains free to develop and market any of its additional compounds.

Mr Scott differentiates Basilea from other start-up companies due to its unique starting position; Basilea combines experience and assets, both in R&D, that would normally take between five and ten years to establish. The technology base in Basilea's fields of activity, are comparable to that of a major pharmaceutical company, yet Basilea retains the focus of a biotech company. Basilea has five novel compounds entering phase I and II clinical trials in the antibacterial, antifungal and dermatology disease areas. They also have two advanced discovery projects for antibacterials and more than ten early discovery projects aimed at maintaining the health of the pipeline. Basilea were assigned the entire antibacterial and antifungal intellectual property portfolio of Roche AG, as well as a substantial portion of Roche's dermatology intellectual portfolio. In addition, Basilea inherited one of the world's largest collections of microbial producer strains and have access to the Roche chemical compound library.

Dr Mann talked in detail about the front-runners in Basilea's drug development portfolio. On the antibacterial and antifungals platform, there are three key players. The first, BAL-5788, is a broad-spectrum iv cephalosporin which has a strong Gram-positive spectrum of activity as well as potent Gram-negative coverage. The drug shows negligible resistance, both in vitro and in animal models. BAL5788 is currently in early phase I clinical testing. The second, BAL-8557, an oral and injectable broad-spectrum azole antifungal agent with high bioavailability, is rapidly cleaved into the active metabolite. The key features of BAL-8557 are the availability of an iv formulation for initial treatment of severe deep mycoses, and an oral formulation for follow-up treatment. It is expected to enter phase I trials in 2002. The final compound, BAL-1198 is a semi-synthetic cyclic depsipeptide representing a new class of broad spectrum antifungal agents. In vitro, BAL-1198 has potent and broad spectrum antifungal activity and, in vivo, it has shown high efficacy, both against systemic candidiasis and *Scedosporium* spp. In vivo, BAL-1198 showed high efficacy against systemic candidiasis and pulmonary aspergillosis in mouse mycosis models.

Of all the compounds, the dermatology candidates have moved furthest along the pipeline. The first of these, BAL-2299, an oral vitamin D3 congener, for the potential

treatment of first line symptoms of plaque psoriasis. It is in phase IIa of development and has shown a good clinical safety profile. The other compound is BAL-4079, an oral retinoid which has published efficacy in chronic hand eczema. A phase II dose finding trial is planned for the fourth quarter of 2001. It is anticipated that, within two years, at least one of the dermatology products will advance into phase III clinical trials.

When questioned on the challenges faced by start-ups in the present financial climate, Mr Scott determined that this would depend on the activities of the individual company. Basilea are presently concerned with in-house product development and thus making a 'clean value' from the company's assets. But, Mr Scott indicated that a company looking for financing at the moment would face a challenge. This opinion was not shared by Dr Kridel from Ferghana Partners who said that providing there is good science, accomplished management and a clear business plan, a company could get venture funding. Dr Kridel added that companies may have to persevere a little longer, but the truth was they'd get a good hearing.

Inex Pharmaceuticals

If Dr Kridel's estimate is correct, then Inex Pharmaceuticals Corp (Canada) represents a success story. Of the three companies interviewed, it is the oldest, and after nine years is still alive and kicking!

Inex Pharmaceuticals is a biotech company, currently employing 130 people, which was founded in 1992 and went public in 1996. It is listed on the Toronto Stock Exchange, the major Canadian equity trading platform.

The company has two technology platforms, liposomal drug delivery of anti-cancer drugs and the development of vaccines based on liposomal carriers. In the case of liposomal delivery, Inex take drugs which have been approved clinically and improve the performance of the drug by putting them inside liposomes. In the case of the vaccine platform, oligosaccharides are placed on the inside and the antigen against which the immune response needs to be created is attached to the outer surface of the vector. According to Dr Madden, Inex has completed proof-of-principle studies with these systems and they are working towards selection of antigens.

On the subject of Inex's pipeline candidates, Tom Madden, Director, Product Development, explained that Inex has one product, Onco-TCS (Inex Pharmaceuticals Corp/Elan Corp plc), which is very close to launch, as well as a number of pipeline candidates that are at a much earlier stage of development. Onco-TCS is currently in a pivotal trial for the indication of non-Hodgkin's lymphoma, the results of which will form the basis for its marketing in the US and Canada. Patient enrollment has been completed, and results from the phase IIa trial showed very good activity in the late stage of the disease. The other candidate generating much excitement at Inex is camptothecin-TCS. Dr Madden explained that the chemical limitations of free camptothecin may be overcome by putting them inside liposomal carriers. He added that the technology used is perfectly suited to delivery of anti-cancer drugs.

When asked who Inex's major competitors were, Dr Madden explained that, in the case of liposomal delivery of anti-cancer drugs, most companies who were major competitors have been bought out by the big pharma companies and that it remained to be seen whether they would continue to develop liposomal technology and develop new drugs based on liposomal drug delivery. However, he thought that, in the case of vaccine development, the market was wide open.

Dr Madden concluded that Inex's focus during the next couple of years would be to bring Onco-TCS to the market; the company needs to generate revenue independent of the equity market. Over the longer term, Inex would like to evolve into a more integrated specialized pharmaceutical company and have its own sales and marketing organization. Since Inex has already signed up a partner to market Onco-TCS, Inex will focus on bringing products currently in earlier stages of development to market.

Summary

Impressions of BPE 2001, expressed by Mr Eastwood, appeared to be representative of views held by all other delegates. BPE is an important date in the calendar for Achillion because it allows the company to try and establish the identity and the expertise of the company in the marketplace and gives them the opportunity to have one-to-one meetings, not only with larger companies, but also with smaller companies to accelerate and enhance their efforts. Mr Eastwood said that, for Achillion, BPE is the key meeting of the year to do some corporate branding and to have discussions concerning strategic collaborations.

The website for this meeting can be found at
http://www.techvision.com/biopartnering_europe/index.html