

EDITOR'S CHOICE

The end of the beginning

Diseases of the CNS are some of the least well understood – or treated – by man. But progress is being made. Newly formed Ellipsis Neurotherapeutics has closed a series A round to fund development of its lead compound, which targets the beta amyloid tangles in Alzheimer's disease (p8). Meanwhile, Psychiatric Genomics has uncovered a new genetic basis for schizophrenia (p12).

Don't leave it too late

The heady days of early stage biotech-pharma deals have not passed. A new agreement between Roche and Pharmasset may be for a research-stage candidate, but the terms are more like those agreed for a Phase II. The biotech stands to make up to \$300M if all goes to plan (p3).

Antibody departure

Antibodies are big news. The stock tables are boosted by clinical antibody successes (p6–7) and companies such as MacroGenics are moving towards the clinic with their antibody products (p10); however, there are also new developments. Symphogen has brought in a series C round to develop its symphobodies (p9) and Domantis has struck a deal to co-develop dAbs – domain antibodies (p2).

Il n'est pas au revoir

French biotech Synt:em may be merging with a US company, but it's not time for goodbye. Synt:em hopes its strategic decision will serve as an example to other French biotechs (p4). Meanwhile, two such firms are making progress: Neovacs and TxCell have both raised funds (p9).

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BVV's editor is changing. Please send future communication to Sarah Walkley on sarah.walkley@informa.com

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KAI's \$28M series A

KAI Pharmaceuticals has added another \$11.0M to its series A financing round, bringing the total raised since inception to \$28.0M. Participants in the round included Skyline Ventures, InterWest Partners, Intersouth Partners and Delphi Ventures, plus Thomas Weisel Venture Partners and MDS Capital in the latest and final closing.

KAI (South San Francisco, CA) recently appointed Steve James as president and CEO. According to James, the latest financing will support clinical and preclinical development into 2006, and provides the resources needed to fund the recently initiated Phase I/II trial of lead candidate, KAI-9803, a small peptide that has been fast tracked by the FDA for the treatment of reperfusion injury following acute myocardial infarction.

"I decided to join KAI because this really is a unique company – it's young but it's maturing rapidly. This is because our scientific founder (Dr Daria Mochly-Rosen from Stanford University) took the technology quite far in an academic setting before approaching VCs," James told *BVV*.

"We hope to finish the Phase I/II trial by the end of 2005. Whether or not we partner the product is a question that we will constantly re-evaluate as we undertake its clinical development. Right now the plan is to conduct Phase I/II ourselves, and then look for a partner when we reach the pivotal trial stage owing to the expense involved."

KAI is developing therapeutics based upon modulation of specific protein kinase C (PKC) isozymes. KAI-9803 targets a PKC isozyme called delta-PKC, which has been found to activate a cascade of events causing cell injury and death during reperfusion. Reperfusion injury occurs when myocardial and endothelial cells undergo necrosis and apoptosis after the reintroduction of blood flow to the ischaemic areas following heart attack. Selective inhibition of the delta-PKC isozyme by KAI-9803 prevents damage to the mitochondria and inhibits both necrosis and apoptosis during reperfusion. KAI reports that in preclinical studies, treatment with KAI-9803 resulted in a 70% reduction in infarct size, an improvement in heart function, restoration of intracellular energy generation, and protection of the cells.

The Phase I/II trial, identified as DELTA MI, will assess the safety and efficacy of KAI-9803 for injection into patients undergoing urgent angioplasty (with or without stent placement) for acute myocardial infarction. This randomised, double-blinded, placebo-controlled study will evaluate increasing doses of KAI-9803. Outcome measures will include clinical endpoints such as heart failure and death, as well as surrogate measures of infarct size, myocardial function, and myocardial perfusion.

In addition to looking at the treatment of reperfusion injury following non-predictive ischaemic events such as heart attacks and strokes, KAI is studying the prevention of reperfusion injury following periods of predictive ischaemia, for example following transplant surgery or surgical procedures such as CABG. "We are also studying the role of PKC in angiogenesis, and working to inhibit abnormal blood growth in ophthalmic disorders such as diabetic retinopathy and age-related macular degeneration (AMD). We are also interested in interrupting the blood supply to tumours. With these programmes we will look for partners after early [preclinical] proof-of-concept," concluded James. – *SV-J*

Series A untangles Alzheimer's

Newly formed **Ellipsis Neurotherapeutics** has closed a Can\$6.0M (\$5.0M) series A round of financing that will fund preclinical and Phase I studies of its lead compound in Alzheimer's disease.

Ellipsis Neurotherapeutics (ENI; Toronto, ON), incorporated in September, has taken over all the Alzheimer's disease programmes from Ellipsis Biotherapeutics (EBI). EBI still exists; it has reformed most of its core business and is concentrating on its SNP genotyping technology as well as some therapeutic programmes in inflammatory bowel disease and rheumatoid arthritis, ENI's director of operations, Richard Wintle, told *BVV*.

Prior to this round, ENI had raised cash through private financing: a seed round from the Canadian Medical Discoveries fund – who participated in the current round – and some angel investors. The company had also received funding from Orchid Biosciences as part of a collaboration.

The new round was prompted by the need to access "enough capital to drive us through discovery", explained Wintle. "We're about to embark on our final preclinical FDA-enabling trials and Phase I. This round should be enough to take us through those."

...controversial

ENI's lead is an orally available small molecule compound for the treatment and prevention of Alzheimer's disease. After oral administration, the molecules are transported across the blood-brain barrier where they act to break down established beta amyloid plaques and prevent the formation of new ones. However, a causal link between the plaques and Alzheimer's disease has not been scientifically proven. "It will be controversial for a while," Wintle predicted. "What we can say is we know our compound works *in vitro*. This disaggregation of beta amyloid fibrils is mirrored *in vivo* and increases life span and improves behavioural and cognitive functions. It's compelling evidence the plaques are a reasonable target."

ENI hopes to file an IND within 12–18 months' time. Beyond Phase I, the company will either adopt the traditional model of out-licensing its compound to a large pharma partner or, if it gets financing, do the trials itself.

Behind the lead is a series of molecules in the same class. "There are a couple that are being developed in related areas of neurodegenerative dementia. There are a lot of diseases where amyloid pathology exists," he commented. – *MG*

■ Bicoll in profit after three years

Sino-German biotech **Bicoll** (Shanghai/Munich) has generated its maiden after-tax profit in only its third year of operations. The firm, which specialises in high-tech natural product chemistry, has five strategic customers for its optimised small molecule libraries (known as Bilobac N) for drug discovery. Using its technology, including prediction toolbox Bipreselect, Bicoll tests and develops its libraries for fees and milestone payments as well as working on in-house projects (*BVV* Vol 17, No 17, p13). This financial milestone places Bicoll "in a stable position from where we can target our second phase of growth to release the full potential of the company", commented managing director Dr Kai Lamottke.