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Positive Phase III Results May Mean Big Payday for Ariad

By Catherine Shaffer
BioWorld Today Contributing Writer

Top-line results from a Phase III trial showed that Ariad Pharmaceuticals Inc.'s ridaforolimus has promise as a treatment for metastatic soft-tissue or bone sarcoma. In the trial designated SUCCEED, carried out under a special protocol assessment granted by the FDA, there was a 28 percent reduction in the risk of progression compared to placebo, and a 21 percent (or 3.1 week) improvement in progression-free survival.

Ariad's stock (NASDAQ:ARIA) surged 34 percent Tuesday, as investors anticipated a successful payday resulting from the future approval of ridaforolimus. Shares closed at \$7.03, up \$1.78.

"This is the largest and most comprehensive trial completed to date in patients with soft-tissue and bone sarcoma," said Harvey J. Berger, Ariad's CEO, during a

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Domain Inks \$180M Early Stage GPCR Deal with Merck Serono

By Nuala Moran
BioWorld Today Correspondent

Domain Therapeutics SA sealed a collaboration potentially worth €134 million (US\$179.6 million) plus royalties for a discovery-stage program targeting a G protein-coupled receptor (GPCR) implicated in Parkinson's and other neurodegenerative diseases.

The deal with Merck Serono SA is the first significant endorsement of the company's platform technology for discovering compounds that act on notoriously hard to handle GPCR targets. "It's also a very important validation of the business model of looking to out-license optimized leads, but on very difficult targets," Pascal Neuville, CEO of the Strasbourg, France-based company told *BioWorld Today*.

On signing, Domain received a €2 million up-front payment, with the remaining €132 million payable against

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Rube Goldberg SNP

Two-Hit Theory of Damage May Apply to Heart Failure as Well

By Anette Breindl
Science Editor

Researchers reported new insights this week into an SNP that shows its carriers are at increased risk of developing heart failure. The protein that contains the SNP within its sequence does not affect the risk of heart failure – and the nearby, linked protein that does affect heart failure risk is not expressed in the heart. Instead, it codes for a chloride channel that is expressed in the kidneys.

Besides suggesting new ways to treat those at risk for heart failure, the study also showed the limitations of using microarrays to identify risk genes. A microarray that only contains certain SNPs, Gerald Dorn told *BioWorld Today*, "doesn't tell you, 'This is it.' It tells you, 'Look here!'"

"Our approach," Dorn said "is to use microarrays to get a view of the genome from 30,000 feet." But "you can't just

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NewCo News

Mimetogen Launches Phase II Trial of Topical NGF in Dry Eye

By Vicki Brower
BioWorld Today Contributing Writer

Focusing on ophthalmic diseases with its portfolio of small-molecule nerve growth factor (NGF) mimetic compounds, Mimetogen Pharmaceuticals Inc. is capitalizing on a large, relatively underserved medical market that is growing at about twice the rate of the general pharmaceutical sector: ophthalmology.

"Historically, eye diseases have been treated with potions," said Garth Cumberlidge, CEO and co-founder. "It is only in the past 10 years that biotech has begun concentrating on ophthalmic diseases." It is an area of research that a few biotechs and a growing number of pharmas now are entering, he added.

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Mimetogen

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Recent research by Mimetogen co-founder Uri Saragovi, and others indicated that NGF, a naturally occurring protein in the eye, plays a key role in ocular disease and health.

The 5-year-old venture-backed company began its first clinical trial in November with lead compound MIM-D3 in dry eye. Mimetogen jumped right into a Phase II study because of excellent safety data in animals, Cumberlidge told *BioWorld Today*.

The Phase II randomized, double-blind, placebo-controlled multicenter U.S. trial is enrolling 150 patients with moderate-to-severe dry eye syndrome, a condition which may be caused by a number of different conditions and produces discomfort, visual disturbance and potentially can damage the eye surface.

The study will test efficacy, tolerability and safety of the drug, administered via eye drops. The study should run until midyear, Cumberlidge said.

After the study concludes, the company expects to meet with the FDA, hopefully by the end of this year. Its aim is to out-license or co-develop MIM-D3 after the Phase II trial is completed.

MIM-D3 is a small cyclic mimetic of NGF, which is a mucin secretagogue designed to act with specificity to quiet inflammation and restore normal lacrimal gland function. In patients with dry eye, mucin is one of the elements in tears that is deficient.

The market for dry eye in the U.S. is about 25 million to 30 million, with equal numbers in Canada and Europe, Cumberlidge said.

There is currently only one drug approved to treat this condition: a topical form of the anti-inflammatory cyclosporine, which has general anti-inflammatory properties. Worldwide, there is an \$8 billion to \$12 billion market for chronic dry eye, with treatment generally long-term, Cumberlidge noted.

A virtual company with four full-time employees, Mimetogen is based in Montreal, home to McGill University and the Lady Davis Institute for Medical Research, where co-founder Saragovi developed the company's neurotrophin mimetic technology that forms the basis of its three ocular disease programs.

Mimetogen's compounds mimic the action of natural receptor ligands of NGF and other neurotrophins. Its drugs are the first small-molecule tyrosine kinase receptor agonists of neurotrophins to be developed, Cumberlidge said. In its other forms, NGF does not have good bioavailability and is expensive to produce, he added.

Mimetogen's second compound is in preclinical testing for glaucoma. That drug is a different formulation of MIM-D3 and is designed to act at the back of the eye as a neuroprotective agent. The company is seeking a partner to take that drug into the clinic.

The as-yet-undesignated glaucoma compound aims to

protect the retinal ganglion cells located in the optic nerve, which die in glaucoma. "Not all patients with glaucoma develop higher intraocular pressure (IOP)," Cumberlidge said. "But for patients with increased IOP, this compound also significantly enhances the neuroprotective effects of IOP-lowering drugs currently used to treat glaucoma."

The drug is meant for administration to the back of the eye, but the exact details of how it will be given have not yet been determined.

So far, Mimetogen has conducted proof-of-concept preclinical studies for the glaucoma drug, which show that it binds to the same receptor as MIM-D3 but on retinal ganglion cells in the back of the eye, protecting them from death.

There are about eight to 10 drugs for glaucoma, which has a huge and growing market with the aging population. Pfizer Inc.'s Xalatan (latanoprost) alone posts \$1.7 billion in sales.

Elsewhere in its pipeline, Mimetogen is developing a compound to treat retinitis pigmentosa.

Founded in 2005, Mimetogen has raised less than \$10 million in three financings, Cumberlidge said. It conducted a Series A financing in March 2006 with Boston's VIMAC Milestone Medica Fund and MSBi Capita of Montreal, and an equity financing in 2009 co-led by VIMAC and Montreal's iNovia Capital.

The firm keeps its burn rate low by conducting all of its R&D work at McGill University. ■

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OBESITY REPORT

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