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Startup developing drug for key market

Henrietta biotech firm using a compound from URMC research team

By WILL ASTOR

A local biotech startup is developing a drug to combat neurocognitive disorders, including dementia and cognitive impairment associated with Parkinson's disease, multiple sclerosis and HIV-AIDS.

Medications are available to treat symptoms of some of those diseases. But no drugs currently available slow or prevent neural damage that leads to dementia—a fate suffered by half of the AIDS patients kept alive by drug cocktails. An agent that could avert such damage has long been one of the pharmaceutical industry's El Dorados.

WavoDyne Therapeutics Inc., housed at the Lennox Tech Enterprise Center in Henrietta, expects to start next year Phase I clinical trials of a drug based on URMC-099. Developed by University of Rochester Medical Center researchers, URMC-099 is a compound that reins in inflammation-causing enzymes.

Inflammation is a needed bodily process that promotes healing. But uncontrolled it can trigger conditions ranging from heart ailments to dementia.

Alzheimer's disease—the cause of more than half of dementias suffered in the United States—is not among the conditions initially targeted by WavoDyne Therapeutics. Still, URMC-099 or a related compound could be used to develop a drug capable of holding Alzheimer's in check, said Harris Gelbard M.D., leader of the URMC group that developed URMC-099.

Gelbard's team includes Stephen Dewhurst, chairman of microbiology and immunology at the UR School of Medicine and Dentistry and researcher Sanjay Maggirwar.

The key to URMC-099's efficacy lies in its ability to cap inflammation without completely shutting it down, said Gelbard, director of the Center for Neural Development and Disease at URMC.

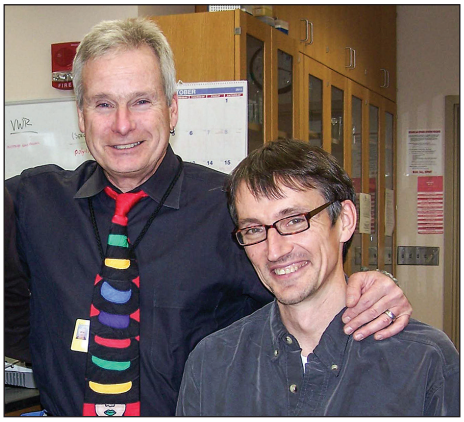

Photo by Kimberly McKinzie

Harris Gelbard M.D. and Stephen Dewhurst led development of URMC-099.

In combatting brain disruptions, an overenthusiastic immune system can defeat an invading agent but cause cognitive impairment in the process, Gelbard said.

Keeping the immune-system response in check without shutting it down potentially could solve a host of problems, allowing the body to defend against diseases and allowing it to heal brain insults while minimizing or eliminating inflammatory collateral damage.

Drug development

While WavoDyne Therapeutics' in-development drug is likely five years away from marketability, founder and CEO Jim New believes URMC-099—and sister compounds covered by the UR-owned patent his firm has licensed—could change the face of medicine.

Describing the licensing deal as "the standard industry split," New declined to elaborate on the arrangement's specifics.

A onetime medicinal chemistry researcher, New moved to the business side of the pharmaceutical industry more than two decades ago. He has handled licensing, business development and mergers and acquisitions for firms including Bristol Meyers Squibb Co., Pfizer Inc., GlaxoSmithKlein PLC and Novartis AG.

In 2002, New co-founded Abrika Pharmaceuticals Inc., a generic drug firm Activis PLC acquired in 2006 in a $235 million deal. He since has been involved in three biotech startups and still heads one of them, AIKO Biotechnology Inc., a Portland, Maine-based firm developing pain-management drugs.

"Of all of them, I think I'm most excited about WavoDyne Therapeutics," New said.

He plans to seek a minimum of $5 million from other investors to finance WavoDyne Therapeutics' immediate next step. New said he would use the money to hire a team of experts familiar with the ins and outs of drug development startups. The company employs two: a communications director and a business development director.

The new hires' mission will be to push URMC-099 through several stages of clinical trials and regulatory approvals as well as to help line up more investors to finance further stages, New said. He is also recruiting board members.

Orleans County native

New is an Albion, Orleans County, native who earned his undergraduate degree, a bachelor of arts degree in biology, from SUNY College at Geneseo. His career as a research scientist and pharmaceutical company executive has taken him to various U.S. cities and to Europe.

Through his travels and relocations, New said, he has held onto a family-owned home in the Rochester area and plans to base himself here for the foreseeable future while he concentrates on WavoDyne Therapeutics.

Courted last year by UR's technology transfer office as a possible developer of a different drug, New decided against backing the drug but came across Gelbard's work. A light bulb went off, but New at first was cautious.

"I spent two months doing my own due diligence before I spoke to Handy," said New, using Gelbard's nickname. "When we finally met, we got along really well, which is important."

Gelbard now serves as chairman of WavoDyne Therapeutics' Scientific Advisory Board. Other members include Dewhurst

and Howard Gendelman M.D., chairman of the University of Nebraska Medical Center Department of Pharmacology and Experimental Neuroscience.

URMC-099's development began with work Gelbard has been doing since 1992 on HIV-associated neurocognitive disorders, a condition known as HAND. He also has investigated similar disorders that can affect elderly post-operative patients, sometimes temporarily and sometimes permanently impairing cognition and memory.

New's enthusiasm for URMC-099 and its sister compounds partly lies in his appreciation of the elegance of Gelbard's work. But he also is beguiled by the drug's commercial possibilities.

"We happen to believe he's hit pay dirt," New said.

An indication of the WavoDyne Therapeutics drug's commercial potential might be inferred from Biogen Inc. CEO George Scangos' announcement that the drug company plans to pump $2.5 billion into development of aducanumab, a new Alzheimer's drug. The move came after some success in a small, early stage clinical trial and an expectation it might generate sales of $14.5 billion if further trials turn out.

A possible damper on Scangos' optimism: Drug makers, including Biogen, previously have announced early success for nine Alzheimer's drugs only to have them sputter out when later stage clinical trials found them less effective than first thought or unsafe for widespread use.

New is less willing than Gelbard to speculate on a URMC-099-related compound's possible application as an Alzheimer's treatment.

"Alzheimer's is not part of the current investigations," he said.

His reticence is more sparked by a desire to avoid spooking potential investors by an association with the high failure rate of past Alzheimer's drug efforts than by doubts about the drug's capabilities, however, New conceded.

Gelbard is less inhibited. He believes URMC compounds could prove effective in fighting not just neurocognitive disorders but a panoply of inflammation-linked diseases—rheumatoid arthritis and heart disease, to name two.

A plus, but also a possible minus for the URMC-099 drug, is Gelbard is unique in pursuing the inflammation-inhibiting line of research.

That means WavoDyne Therapeautics does not have to worry about competitors beating it to the punch. But the reason there are none is other researchers abandoned such pursuits some eight years ago after a similar effort crashed.

A wholly owned subsidiary of Israel-based Teva Pharmaceuticals, Cephalon Inc., had followed a similar path, developing a compound cep-1347 that also reins in inflammation-causing enzymes.

Teva rushed cep-1347 into clinical trials as a treatment for Parkinson's, generating a fair amount of excitement in scientific circles and among stock analysts. But in 2005, it had to abandon the trials.

The reason: Partly through experiments Gelbard devised, it became evident that even though cep-1347 could inhibit MLK enzymes' inflammatory effects, it was never able to reach the brain and thus was ineffective.

Cep-1347's failure cast a pall over MLK-inhibitor research, Gelbard said. Only he and his team kept at it. Ingenious methods Gelbard devised to test cep-1347 proved to be an essential tool in developing the URMC compounds, New said.

New, who wonders what Teva officials might have been thinking when they prematurely put cep-1347 into clinical trials, plans to follow a more cautious path.

The eventual end game he foresees for the company is the same goal most pharmaceutical startups aim for: A lucrative deal granting rights to mass produce and mass market a URMC-099 drug to a major drug company.

"We're not ready to talk about that now," New cautions. "That's at least five years in the future."

Still, he added, when that day comes the company will be ready. Patents are in hand covering URMC-099 and any sister compounds that might be developed for the United States, China and New Zealand, and pending for Canada, Europe, Australia and Japan, he said.

And, added New, as Gelbard's research continues to uncover more URMC molecules, "I'm sure we'll negotiate rights to those patents too."

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