

# Ligand Licenses DARA Program to Retrophin

**Potential for over \$75 million in milestone payments to Ligand plus royalties**

**Retrophin plans to develop DARA for rare nephropathies and other indications**

SAN DIEGO & NEW YORK-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** and **Retrophin, LLC** announced that the companies have entered into an agreement in which Ligand has licensed rights to DARA (a Dual Acting Receptor Antagonist of Angiotensin and Endothelin receptors) to Retrophin. Under the terms of the agreement, Ligand will receive a net upfront payment of \$1 million, and may receive, net of amounts owed to third parties, over \$75 million in milestone payments based on clinical and regulatory progress as well as 9% in royalties on potential future worldwide sales by Retrophin.

“DARA has the promise to be a significant medicine, and we are excited to partner with Retrophin to advance the development of the program and bring it closer to patients in need,” said John Higgins, President and Chief Executive Officer of Ligand Pharmaceuticals. “This is an attractive deal for Ligand and our shareholders. We have partnered DARA with a team that has great credentials, is highly motivated to advance the program and has a compelling development plan. This is another valuable asset in our late-stage portfolio.”

Retrophin intends to develop DARA for orphan indications of severe kidney diseases including Focal Segmental Glomerulosclerosis (FSGS) as well as conduct proof-of-concept studies in resistant hypertension and diabetic nephropathy. Certain patient groups with severely compromised renal function exhibit extreme proteinuria resulting in progression to dialysis and a high mortality rate. DARA, with its unique dual blockade of angiotensin and endothelin receptors, is expected to provide meaningful clinical benefits in mitigating proteinuria in indications where there are no approved therapies.

“We are pleased to execute this agreement with Ligand as it is consistent with our mission of improving the lives of patients with rare and life-threatening diseases,” said Martin Shkreli, Chief Executive Officer of Retrophin, LLC. “An estimated 50,000 patients in the United States are afflicted with FSGS and DARA has the potential to make a meaningful difference in their lives. DARA may help patients who are at a high risk of losing their kidneys to their diseases by delaying the progression and reversing severe markers of kidney damage such as proteinuria. We will work to advance DARA into a pivotal trial as quickly as possible, as these desperate patients currently have few treatment options available to them.”

## **About DARA**

Ligand acquired DARA (PS433540) in its acquisition of Pharmacoepia in December 2008. The compound possesses two clinically validated mechanisms of action that selectively

block two potent vasoconstrictor and mitogenic agents, angiotensin II and endothelin 1, at their respective receptors. In Phase IIb studies for hypertension completed in 2009, DARA was found to be safe and well tolerated, and demonstrated statistically significant greater reduction in blood pressure compared with placebo and with irbesartan.

The 261-patient, randomized, double-blind, placebo- and active-controlled study evaluated safety and efficacy at three different doses in subjects with Stage 1 and Stage 2 hypertension over 12 weeks of treatment. The high dose of DARA produced a statistically significantly greater reduction in blood pressure than the active comparator, irbesartan, which was tested at its highest approved dose.

### **About Retrophin, LLC**

Retrophin, LLC is a privately-held New York-based, biotechnology company focused on discovering and developing treatments for rare and life-threatening diseases. Retrophin is currently developing treatments for Duchenne muscular dystrophy, spinal muscular atrophy, cystic fibrosis and myotubular myopathy. Retrophin's lead internally discovered compound, RE-001, is a protein replacement therapy for Duchenne muscular dystrophy. Retrophin will hereinafter refer to DARA as RE-021 and intends to initially develop the drug for rare nephropathies, including Focal Segmental Glomerulosclerosis. Retrophin's Series A financing was led by MSMB Capital and several current and former senior executives at global pharmaceutical and healthcare companies.

### **About Ligand Pharmaceuticals**

Ligand is a biopharmaceutical company with a business model that is based upon the concept of developing or acquiring royalty revenue generating assets and coupling them to a lean corporate cost structure. Ligand's goal is to produce a bottom line that supports a sustainably profitable business. By diversifying the portfolio of assets across numerous technology types, therapeutic areas, drug targets and industry partners, we offer investors an opportunity to invest in the increasingly complicated and unpredictable pharmaceutical industry. We believe Ligand has assembled one of the largest and most diversified asset portfolios in the industry with future revenue-generating potential. These therapies address the unmet medical needs of patients for a broad spectrum of diseases including hepatitis, muscle wasting, Alzheimer's disease, dyslipidemia, diabetes, anemia, asthma, rheumatoid arthritis and osteoporosis. Ligand's Captisol platform technology is a patent protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. Ligand has established multiple alliances with the world's leading pharmaceutical companies including GlaxoSmithKline, Merck, Pfizer, Eli Lilly & Company, Baxter International, Bristol-Myers Squibb, Celgene, Onyx Pharmaceuticals, Lundbeck Inc., The Medicines Company, Curis, Inc. and Rib-X Pharmaceuticals. Please visit [www.captisol.com](http://www.captisol.com) for more information on Captisol. For more information on Ligand, please visit [www.ligand.com](http://www.ligand.com).

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### **Caution Regarding Forward-Looking Statements**

This news release contains forward looking statements by Ligand and Retrophin that involve risks and uncertainties and reflect Ligand's and Retrophin's judgment as of the date of this

release. Actual events or results may differ from Ligand's or Retrophin's expectations. For example, there can be no assurance that DARA or any product in the Ligand or Retrophin pipelines will be successfully developed, that any of the milestone triggers will be achieved, that regulatory approvals will be granted, that patient and physician acceptance of these products will be achieved, that final results of human clinical trials will be consistent with any interim results, that final results will be supportive of regulatory approvals required to market products or that any revenue will be achieved from this partnered program. In addition, if Retrophin chooses to sub-license the program the actual financial amounts Ligand receives may be lower than licensing terms specified in this press release. Additional information concerning these and other risk factors affecting Ligand's business can be found in prior press releases available via [www.ligand.com](http://www.ligand.com) as well as in Ligand's public periodic filings with the Securities and Exchange Commission at [www.sec.gov](http://www.sec.gov). Ligand and Retrophin disclaim any intent or obligation to update these forward-looking statements beyond the date of this release. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

Photos/Multimedia Gallery Available: <http://www.businesswire.com/cgi-bin/mmg.cgi?eid=50173816&lang=en>

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