

## Rosetta Genomics Provides Product Pipeline Update

*Leveraging world-class microRNA biomarker platform to advance proprietary technologies in diagnostics and therapeutics*

*Plans to launch one new product each year commencing in 2015*

**PRINCETON, N.J. and REHOVOT, Israel (March 12, 2014)** – Rosetta Genomics Ltd. (NASDAQ: ROSG), a leading developer and provider of microRNA-based molecular diagnostics, announces details regarding the Company’s plans to advance its proprietary microRNA platform technologies in diagnostics and therapeutics.

“Rosetta Genomics continues to set the pace in developing and commercializing microRNA-based technologies. We have the most validated microRNA biomarker platform with approximately 50 peer-reviewed publications relating to our platform and have recently rejuvenated our research and development efforts to leverage this leading and versatile platform,” stated Kenneth A. Berlin, President and Chief Executive Officer of Rosetta Genomics. “We are moving forward on a variety of important projects to provide clinicians with better tools to improve patients’ lives in a number of areas of unmet medical need with an aim to commercialize one new assay per year commencing in 2015.”

### **Diagnostics**

Building upon the Company’s four current commercial diagnostic assays, Rosetta is advancing several new diagnostic assays starting with our lead assay for the differential diagnosis of thyroid neoplasia. In addition, we are evaluating several assays for other cancers as well as assays for non-invasive diagnosis of chronic kidney rejection; the early diagnosis and risk stratification of heart failure patients; and the early diagnosis and differentiation of Alzheimer’s disease.

#### *Thyroid neoplasia differential diagnosis*

Rosetta is discovering tissue-based microRNA biomarkers in order to develop a new assay for the differential diagnosis of indeterminate thyroid Fine Needle Aspirate (FNA) samples.

An estimated 4% to 7% of the general population develops nodules in the thyroid that can be felt on examination, though fewer than 10% are malignant. An FNA to obtain tissue for analysis is the standard technique for detecting cancer. It is estimated that nearly 500,000 FNAs are performed each year in the U.S. and approximately 740,000 are performed annually in Europe. Interpretation of FNA samples is not always straightforward, leading to an indeterminate result in up to 30% of the samples. Many patients with indeterminate results are sent to surgery as a

precaution, despite the fact that the majority of these cases are benign. This exposes patients to unnecessary surgical risk and costs the system hundreds of millions of dollars.

Rosetta is developing a thyroid neoplasia assay that has the potential to improve clinical management in a cost-effective manner by reducing the number of unnecessary surgeries.

Results from initial studies demonstrated that microRNA expression levels can differentiate malignant nodules from benign nodules, and also demonstrated the ability to extract and profile microRNAs from thyroid FNAs, in various sample types and in a way consistent with common clinical practices. Rosetta is currently commencing larger studies and anticipates launching this assay prior to the end of 2015.

#### Other cancers

In addition to the thyroid neoplasia diagnostic, Rosetta is seeking to expand its oncology diagnostic testing services to leverage its growing commercial infrastructure. The Company is evaluating the results of two studies relating to an assay for bladder cancer risk of invasiveness. In addition, the Company is evaluating results from an earlier study relating to predicting treatment response in ovarian cancer patients. Finally, in the area of breast cancer, Rosetta is evaluating the unmet needs here and is in discussion with potential collaborators regarding the development of assays that could aid clinicians in patient management. Diagnostics for each of these indications are important, as the information they supply will directly affect treatment choices and patient outcomes. Rosetta is evaluating next steps in each of these important oncology indications as it seeks to leverage the investments it is making in its commercial oncology infrastructure over a larger number of cancer products with the goal of launching at least one assay for an oncology indication prior to the end of 2016. In addition, our core research continues to enhance and expand the range and clinical utility of our current products for better diagnostic guidance of therapeutic strategies in difficult metastatic cancers, and lung and kidney cancers. These efforts leverage methodological advances from new test research as well as our clinical lab experience.

#### Non-invasive diagnosis of chronic kidney rejection

Kidney transplantation is widely accepted as the treatment of choice for end-stage renal disease. The number of patients receiving renal replacement therapy worldwide is estimated at more than 1.4 million and is growing by approximately 8% annually. Advances in post-transplant care including the introduction of new potent immunosuppressive drugs have led to a dramatic decrease in acute kidney rejection. However, long-term stable graft function remains elusive, and in the past decade, progressive chronic deterioration, or chronic rejection, and the resulting inevitable graft loss has become a major problem in organ transplantation.

The incidence of rejection is high, at a rate of 30% of all grafts in the first post-transplant year, progressing to 50% in year five and reaching nearly 100% of functioning grafts 10 years after transplantation. Chronic rejection is currently diagnosed only by histological examination of a tissue sample obtained by biopsy, which is expensive, unpleasant for the patient and unsuitable for serial clinical use due to the risk of bleeding and other complications. Furthermore,

morphological changes may be patchy, difficult to interpret and subject to observer bias. Therefore, it is widely accepted that measurement of chronic graft injury is imprecise.

Rosetta is commencing work on developing blood- or urine-based microRNA biomarkers to advance a new diagnostic test for chronic kidney rejection. This non-invasive test has the potential to enable diagnosis at an earlier stage when this information could alter the choice of therapy and, ultimately, improve outcomes. Rosetta expects to complete development of and launch this assay in 2017.

### Heart failure

Rosetta is evaluating results from its two studies relating to blood-based microRNA biomarkers to advance a new diagnostic assay for the early diagnosis of heart failure (HF) and refined risk stratification of patients following myocardial infarction (MI, or heart attack).

HF is the most prevalent disease in the western world and is the only cardiovascular disease whose prevalence continues to rise. The worldwide prevalence of HF is estimated to be 23 million; with 6.6 million in the U.S. HF is one of the most expensive diseases in western countries, with costs to the U.S. healthcare system estimated at \$31 billion in 2012.

The Company has two supportive studies published. The first was published in the *European Journal of Heart Failure* (Goren et al., 2012) and showed that elevated serum levels of specific microRNAs identify HF patients. The second was published in the *American Journal of Cardiology* (Goren et al., 2013) and showed a significant reduction in the level of a specific microRNA (miR-150) in platelets of HF patients that also suffered atrial fibrillation (heart rhythm disorder).

Rosetta is planning to conduct additional studies aimed at further examining the expression of the identified microRNA biomarkers in various blood fractions and anticipates launching a new assay in HF in 2017.

### Alzheimer's disease

New and expensive drug therapies are expected to increase the need for and value of early diagnosis in Alzheimer's disease (AD). Rosetta has profiled serum samples of AD patients in comparison to serum samples healthy controls and identified that several microRNAs in AD blood maintained their direction when compared between AD brain and brain cancer tissue. Rosetta plans to conduct a proof-of-concept study in additional cohorts in order to profile differentially expressed microRNAs in the cerebrospinal fluid of AD patients compared with normal donors using deep sequencing. The Company expects to advance studies relating to this project in 2014.

## **Therapeutics**

microRNAs represent potential targets for the development of novel drugs and there are now at least two drugs targeting microRNAs that are in clinical studies by other companies. Rosetta's therapeutics pipeline has three distinct projects including one aimed at developing a microRNA-based therapeutic for cytomegalovirus (CMV) infection and two government-

assisted projects focused on the development of novel technologies for the delivery of microRNA-based therapeutics.

### CMV therapeutic

microRNAs are abundant among various organisms including DNA viruses of the herpes virus family (e.g., CMV). Viral microRNAs may directly regulate viral genes, could target host genes and are involved in viral replication and in assisting the virus evade the host immune system.

Human CMV is a widespread pathogen that infects the majority of the world's population by early adulthood. Human CMV can be the cause of morbidity and mortality in populations with immature or compromised immune systems. With 30,000 infants born with congenital CMV annually in the U.S., it is the leading viral cause of birth defects where infection of neonates causes deafness and mental retardation. CMV affects 90-95% of HIV-infected patients and is the major cause of retinitis and blindness in AIDS patients. Human CMV contributes to graft loss in bone marrow and solid organ transplants, causes disease in cancer patients receiving immunosuppressive chemotherapy and likely contributes to age-associated immunosenescence.

There is a pressing medical need for novel therapeutics for CMV infection as there is no vaccine to prevent it and FDA-approved drugs for the treatment of CMV suffer from low bioavailability, toxicity and the formation of resistant viruses. According to GlobalData, the CMV drug market is expected to grow to \$1.2 billion by 2019 from \$685.5 million in 2011.

Rosetta is in the discovery stage of a project aimed at developing a microRNA-based therapeutic for the treatment of human CMV infection. Rosetta's researchers have identified several viral microRNA candidates and have prepared a library of microRNA mimetics as well as specific inhibitors of these microRNAs. The Company is studying the effect of the library compounds on viral replication and infectivity.

Rosetta anticipates selecting a lead candidate in the third quarter of 2014 and is considering various alternative routes to market, including through or with third parties, for further preclinical and clinical development.

### Rimonim and Magnetron projects

The Rimonim and Magnetron projects aim at developing novel technologies for the effective delivery of microRNA mimetics for therapeutics. Both projects are supported by the Office of Chief Scientist at the Ministry of Industry, Trade and Labor of the State of Israel (OCS). If successful, Rosetta will seek to use these technologies for future internal projects as well as to partner them with other therapeutics companies.

"Our microRNA platform technologies are extremely versatile and we have developed a robust pipeline of opportunities. Our ongoing goal is to leverage our leading platform in microRNA biomarker technology to bring innovative diagnostics and therapeutics to patients in need, while creating the potential for new revenue sources for the Company. We plan on building our robust pipeline to leverage our investments in oncology, while expanding our microRNA technologies into much larger indications such as heart failure and Alzheimer's disease. We

will invest and advance programs on our own in certain indications and expect to collaborate, partner or license our technologies and products in others," concluded Mr. Berlin.

### **About Rosetta Cancer Testing Services**

Rosetta Cancer Tests are a series of microRNA-based diagnostic testing services offered by Rosetta Genomics. The Rosetta Cancer Origin Test™ can accurately identify the primary tumor type in primary and metastatic cancer including cancer of unknown or uncertain primary (CUP). Rosetta Mesothelioma Test™ diagnoses mesothelioma, a cancer connected to asbestos exposure. The Rosetta Lung Cancer Test™ accurately identifies the four main subtypes of lung cancer using small amounts of tumor cells. The Rosetta Kidney Cancer Test™ accurately classifies the four most common kidney tumors: clear cell renal cell carcinoma (RCC), papillary RCC, chromophobe RCC and oncocytoma. Rosetta's assays are designed to provide objective diagnostic data; it is the treating physician's responsibility to diagnose and administer the appropriate treatment. In the U.S. alone, Rosetta Genomics estimates that 200,000 patients a year may benefit from the Rosetta Cancer Origin Test™, 60,000 from the Rosetta Mesothelioma Test™, 65,000 from the Rosetta Kidney Cancer Test™ and 226,000 patients from the Rosetta Lung Cancer Test™. The Company's assays are offered directly by Rosetta Genomics in the U.S., and through distributors around the world. For more information, please visit [www.rosettagenomics.com](http://www.rosettagenomics.com). Parties interested in ordering the test can contact Rosetta Genomics at (215) 382-9000 ext. 309.

### **About Rosetta Genomics**

Rosetta develops and commercializes a full range of microRNA-based molecular diagnostics. Founded in 2000, Rosetta's integrative research platform combining bioinformatics and state-of-the-art laboratory processes has led to the discovery of hundreds of biologically validated novel human microRNAs. Building on its strong patent position and proprietary platform technologies, Rosetta is working on the application of these technologies in the development and commercialization of a full range of microRNA-based diagnostic tools and therapeutics. Rosetta's cancer testing services are commercially available through its Philadelphia-based CAP-accredited, CLIA-certified lab. Frost & Sullivan recognized Rosetta Genomics with the 2012 North American Next Generation Diagnostics Entrepreneurial Company of the Year Award.

### **Forward-Looking Statement Disclaimer**

Various statements in this release concerning Rosetta's future expectations, plans and prospects, including without limitation, Rosetta's aim to commercialize one new assay per year commencing in 2015, as well as the timeline for launching new assays (including launch of a thyroid neoplasia assay by the end of 2015, launch of an oncology assay by the end of 2016, launch of an assay for chronic kidney rejection in 2017, and launch of an HF assay in 2017), the timing of development of an assay for early diagnosis of AD, the expected growth of the CMV drug market, anticipation of selecting a lead CMV drug candidate in the third quarter of 2014, and Rosetta's development of novel technologies for the effective delivery of microRNA mimetics for therapeutics, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including those risks more fully discussed in the "Risk Factors" section of Rosetta's Annual Report on Form 20-F for the year ended December 31, 2012 as filed

with the SEC. In addition, any forward-looking statements represent Rosetta's views only as of the date of this release and should not be relied upon as representing its views as of any subsequent date. Rosetta does not assume any obligation to update any forward-looking statements unless required by law.

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