





# Mesoblast, Intrexon and ZIOPHARM to Target Cancers

Partnership Combines Mesoblast's Cell and Intrexon's Transgene Technologies

**Melbourne, Australia; Germantown, MD and Boston, MA, USA; October 23, 2013** – Mesoblast Limited (ASX: MSB), a world leader in the development of cell-based biologic products, Intrexon Corporation (NYSE: XON), a leader in synthetic biology, and ZIOPHARM Oncology, Inc. (NASDAQ: ZIOP), a biopharmaceutical company focused on the discovery and development of new cancer therapies, today announced a partnership to develop a new class of cancer therapeutics.

Under the terms of the agreement the companies will combine Mesoblast's proprietary Mesenchymal Lineage Cells (MLCs) with Intrexon's RheoSwitch Therapeutic System<sup>®</sup> (RTS<sup>®</sup>) platform to co-develop complex transgene enabled cell-based treatments for oncology applications. The goal of combining these technologies is to enable development of therapeutic candidates in the treatment of cancer which exhibit both specific tumor targeting characteristics and controlled gene expression. The partnership is a 50/50 collaboration between Mesoblast and Ziopharm, with Intrexon participating through its Exclusive Channel Collaboration with Ziopharm. If successful in feasibility studies targeting lung cancer, the companies expect to form a joint venture to advance development of therapeutic candidates.

Randal J. Kirk, Chairman and Chief Executive Officer of Intrexon Corporation, noted that the initial studies and subsequent joint venture will bring together unique Intrexon technology, Mesoblast cell technology and production expertise and ZIOPHARM development expertise.

"The combination of expertise, technologies and production capabilities represented by Mesoblast, ZIOPHARM, and Intrexon is an excellent example of how collaborations will be expanded into new areas of therapeutic research and development," Kirk said. "Through this program each party has an essential role that is indispensable to its advancement and hopefully to the development of more effective cancer therapies."

Silviu Itescu, Chief Executive Officer of Mesoblast Limited, said: "MLCs modified with RTS<sup>®</sup> technology could be ideal vehicles to deliver therapeutic transgenes to targeted tumors at specific sites in the body. Additionally, our cells can be commercially manufactured to industrial scale for clinical use as they are readily expanded to large numbers in culture, can be used in allogeneic recipients without the need for matching or immunosuppression, and are available cryopreserved for off-the-shelf use.

"By seeking to develop anti-cancer effectors using our proprietary MLCs, we are now expanding the focus of our oncology therapeutic area beyond the field of bone marrow transplants and the treatment of its major complication, acute graft-versus-host disease. If this complex cell plus transgene approach is successful for lung cancers, the technology will be explored for the treatment of other cancers," Itescu said.

In initial studies, ZIOPHARM will leverage Intrexon's <u>UltraVector<sup>®</sup> platform</u> to design and optimize therapeutic gene expression in MLCs to mediate an anti-cancer effect against lung cancer. Intrexon's RTS<sup>®</sup> platform will also be utilized for inducible control over the amount and timing of therapeutic gene expression providing precise regulation for MLC delivered therapies.

Jonathan Lewis, M.D., Ph.D., chief executive officer of ZIOPHARM Oncology, Inc. said, "The ability of Intrexon's RTS<sup>®</sup> platform to induce and regulate expression of anti-cancer effectors through dosing with an activator has been demonstrated in current Phase 2 studies for the treatment of advanced melanoma and breast cancer<sup>1</sup>. By integrating Mesoblast's MLC platform into our therapeutic complex transgene research and development program, we hope to target advanced non-small-cell lung cancer and advance a new field of cell-based therapies."

"Lung cancer represents a significant unmet medical need," said Samuel Broder, M.D., Senior Vice President of Intrexon's Health sector and former Director of the National Cancer Institute. "The co-development of complex RTS<sup>®</sup> regulated transgenes and cell-based therapy into a novel treatment means lung cancer cells could be attacked simultaneously in two dimensions. The first is through the delivery of the modified MLCs to the lung tissue. The second is through Intrexon's proven RheoSwitch<sup>®</sup> technologies in which therapeutic transgenes are expressed under the control of a small molecule activator, permitting MLCs to deliver antitumor proteins to sites where they are needed. Delivery plus expression and regulation should prove to be powerful combination."

According to the United States National Cancer Institute, it is estimated that 228,190 patients will be diagnosed with lung cancer, and nearly 160,000 patients will die of this disease in 2013. Lung cancer is the leading cause of cancer death in both men and women in the United States. Lung cancer causes more cancer-related deaths than the next three most common cancers combined (colon, breast and prostate).

## About Mesoblast Limited

Mesoblast Limited (ASX: MSB; USOTC: MBLTY) is a world leader in the development of biologic products for the broad field of regenerative medicine. The Company's proprietary technologies include its Mesenchymal Precursor Cell and culture-expanded Mesenchymal Stem Cell technology platforms, Dental Pulp Stem Cells, and expanded Hematopoietic Stem Cells. Mesoblast's allogeneic or 'off-the-shelf' regenerative medicine products focus on repair of damaged tissues and modulation of inflammatory responses in conditions with significant

<sup>&</sup>lt;sup>1</sup> ClinicalTrials.gov NCT01397708; NCT01703754

unmet medical needs. The lead product candidates use its mesenchymal lineage cells in four major and distinct areas - systemic inflammatory conditions, cardiovascular diseases, orthopedic diseases of the spine, and oncology conditions. www.mesoblast.com

#### About Intrexon Corporation

Intrexon Corporation (NYSE: XON) is a leader in synthetic biology focused on collaborating with companies in Health, Food, Energy and the Environment to create biologically-based products that improve the quality of life and the health of the planet. Through the company's proprietary UltraVector<sup>®</sup> platform, Intrexon provides its partners with industrial-scale design and development of complex biological systems. The UltraVector<sup>®</sup> platform delivers unprecedented control over the quality, function, and performance of living cells. We call our synthetic biology approach and integrated technologies Better DNA<sup>®</sup>, and we invite you to discover more at <u>www.dna.com</u>.

#### About ZIOPHARM Oncology, Inc.

ZIOPHARM Oncology is a biopharmaceutical company focused on the development and commercialization of new cancer therapies. ZIOPHARM's operations are located in Boston, MA. Further information about ZIOPHARM may be found at <u>www.ziopharm.com</u>.

#### Intrexon Safe Harbor Statement

Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to our plans, objectives and expectations for the development of our business. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.

### ZIOPHARM Forward-Looking Safe Harbor Statement:

This press release contains certain forward-looking information about ZIOPHARM Oncology, Inc. that is intended to be covered by the safe harbor for "forward-looking statements" provided by the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. Words such as "expect(s)," "feel(s)," "believe(s)," "will," "may," "anticipate(s)" and similar expressions are intended to identify forward-looking statements. These statements include, but are not limited to, statements regarding our ability to successfully develop and commercialize our therapeutic products; our ability to expand our long-term business opportunities; financial projections and estimates and their underlying assumptions; and future performance. All of such statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include, but are not limited to: whether palifosfamide, Ad-RTS-IL-12, darinaparsin, indibulin, or any of our other therapeutic products will advance further in the clinical trials process and whether and when, if at all, they will receive final approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies and for which indications; whether palifosfamide, Ad-RTS-IL-12, darinaparsin, indibulin, and our other therapeutic products will be successfully marketed if approved; whether any of our other therapeutic product discovery and development efforts will be successful; our ability to achieve the results contemplated by our collaboration agreements; the strength and enforceability of our intellectual property rights; competition from other pharmaceutical and biotechnology companies; the development of, and our ability to take advantage of, the market for our therapeutic products; our ability to raise additional capital to fund our operations on terms acceptable to us; general economic conditions; and the other risk factors contained in our periodic and interim SEC reports filed from time to time with the Securities and Exchange Commission, including but not limited to, our Annual Report on Form 10-K for the fiscal year ended December 31, 2012, and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2013. Readers are cautioned not to place undue reliance on these forwardlooking statements that speak only as of the date hereof, and we do not undertake any obligation to revise and disseminate forward-looking statements to reflect events or circumstances after the date hereof, or to reflect the occurrence of or non-occurrence of any events.

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