

**ERGONEX** 

**2009 European Orphan Diseases  
Entrepreneurial Company Award**



*“We accelerate growth”*

## 2009 European Orphan Diseases Entrepreneurial Company Award

### Award Description

The Frost & Sullivan Award for Entrepreneurial Company is presented to the small company that demonstrated superior entrepreneurial ability in its industry during the research period. This Award signifies the company's identification of a unique product solution with significant market potential. Additionally, the Award certifies that the company's marketing strategy is sound and poised for success.

### Research Methodology

Entrepreneurial ability is assessed using mostly primary research with top manufacturers and experts in the industry. Frost & Sullivan's analyst teams perform extensive interviews with the company in question to evaluate its business, products, and marketing plan. In addition, primary research with leading manufacturers is performed to benchmark the Award recipient's strategy for growth against established players' strategies. Also considered are elements such as strategic alliances, expected time to market, and the senior management team. Research is also conducted to evaluate and compare the value of the Award recipient's product solution.

### Measurement Criteria

A recipient that is chosen for the Frost & Sullivan Award for Entrepreneurial Company must match the following criteria:

- The company must have fewer than 300 employees.
- The company must have identified a brand new and completely unique product solution.
- The product solution must have significant market potential - at least \$200 million - and a high probability of reaching its potential in the next 2-5 years.
- Financial and employee-based resources to ensure a large probability of success. Financial resources include backing from VCs, IPOs, and funding from large corporate partners.
- Protection from competitors: such as patents, large product development lead time and strategic alliances with key component suppliers.
- Strong plans for marketing: such as strategic alliances for distribution, relationships with key customers, voluminous positive-press in the media and endorsements from industry experts.



The “2009 European Orphan Diseases Entrepreneurial Company Award,” in the European orphan diseases market is presented to Ergonex Pharma GmbH. The Award is in recognition of the company’s impressive display of technological know-how and targeted vision within the orphan diseases market. Ergonex Pharma GmbH integrates the best research and development (R&D) skilled technologists.

### **The Company**

Ergonex Pharma GmbH was founded in 2004 and is headquartered in Appenzell, Switzerland. The organisation focuses on the development and commercialisation of orphan disease therapeutics. With an innovative therapeutic concept on hand, Ergonex Pharma GmbH aims at improving the therapy of distinct orphan diseases.

Since its inception, Ergonex Pharma GmbH has been actively involved in efforts to develop and commercialise orphan disease therapeutics through in-house R&D. Currently, Ergonex Pharma GmbH has a good employee base with experts in the fields of pharmacology and medicine.

### **Unique Value Proposition**

Ergonex Pharma GmbH focuses on refining existing therapies and thereby, improves the standard of care. A multi-disciplinary team of highly inventive scientists work to design products that meet the critical demand of patients. The therapeutic areas of research include pulmonary arterial hypertension (PAH), lung fibrosis and systemic sclerosis.

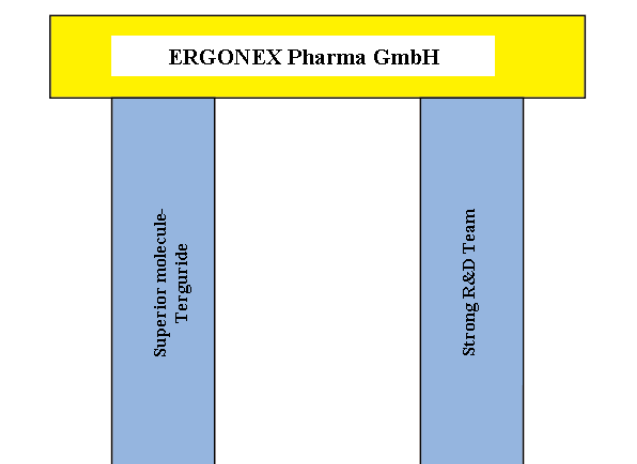
Ergonex Pharma GmbH utilises targeted therapy to combat PAH. The ultimate aim of this organisation is to concentrate on drug candidates that fair better in treating this rare and severe orphan disease. Armed with powerful technologies and superior research & development team, Ergonex Pharma GmbH is all set to emerge as a successful entrepreneurial company. A potential high value generating drug candidate is currently in the clinical trials. Ergonex Pharma GmbH believes that the main drivers of growth are technology and product competitiveness. The US and EU approval for orphan drug status provides a competitive edge over the established market players by offering product solutions that work through pathways, which are not addressed by current therapies and have a strong potential for synergy as well as to introduce a new quality into treatment of PAH.

Furthermore, Ergonex Pharma GmbH is focused on expanding its orphan disease therapeutics portfolio. The strategic move of Ergonex Pharma GmbH in extending the indication of the orphan drug – Terguride from PAH to systemic sclerosis reinforces its commitment in fighting the orphan diseases.

## Pioneering Innovation

Since its inception backing 2004 Ergonex Pharma GmbH continues to draw immense attention from the investment community and this is a testimony to its foundations and success in a relatively short timespan. Ergonex Pharma GmbH is moving closer to its vision of establishing itself as the leader and innovator in developing and commercialising effective drugs for orphan diseases. The R&D spend on innovating drugs has been the major focus for Ergonex Pharma GmbH. Ergonex Pharma GmbH is the pioneer of this novel therapeutic use of the drug - Terguride and this molecule has the potential to create new milestones in orphan disease therapeutics. The orphan drug designation from EMEA and FDA was granted in 2007 and 2008, respectively

Chart I.1 Depicts the pillars of success for Ergonex Pharma GmbH.



Source: Frost & Sullivan

## Overcoming Industry Challenges

Ergonex Pharma's Terguride molecule comes in the most opportune of times when PAH market is growing in a fast pace. Ergonex Pharma GmbH employs its drug candidate with a novel mechanism of action and enhanced properties. It has an attractive tolerability profile, good patient compliance and is cost effective, when compared with existing therapeutic drugs. This molecule offers various advantages including oral availability.

The Terguride molecule scores high on performance and has attracted a lot of interest from the medical community. Clinical studies have proven the safety profile of this drug. Ergonex Pharma GmbH has invested significantly in pharmacological profiling of its drug candidate and in understanding the role of serotonin in PAH and fibrotic disorders. Serotonin has been implicated in the progression of PAH for a long time and recent advances in basic science have identified the crucial role of distinct receptors. Terguride acts as a non-surmountable antagonist on those serotonin receptors, which drive vascular remodeling processes and vasoconstriction in PAH. A therapeutic use of Terguride is presently explored in a multicentric, clinical proof-of-concept study. This drug is likely to witness a fast adoption among the medical community owing to its patient friendliness and is expected to gain wide acceptance among healthcare professionals.

## **Best Practices**

Ergonex Pharma's strategy looks at developing the best drugs by defining and developing novel uses of existing products and leveraging it to produce highly differentiated products that are high on returns. This unique business model has an edge over competition whereby investment in this model is less compared to researching a new molecule. Ergonex Pharma GmbH believes in global partnerships with pharmaceutical organisations on all continents. Ergonex Pharma's strategy is to work on high probability products that have a high impact on the patient population.

## **Conclusion**

Ergonex Pharma GmbH has renowned scientists to launch innovations in the treatment of PAH. Terguride has the potential to transform the orphan disease therapeutics market. The Terguride molecule is likely to meet certain unmet demands of the orphan diseases community. These factors make Ergonex Pharma GmbH a worthy recipient of the "2009 European Orphan Diseases Entrepreneurial Company Award."

## About Best Practices

Frost & Sullivan Best Practices Awards recognise companies in a variety of regional and global markets for demonstrating outstanding achievement and superior performance in areas such as leadership, technological innovation, customer service, and strategic product development. Industry analysts compare market participants and measure performance through in-depth interviews, analysis, and extensive secondary research in order to identify best practices in the industry.



## About Frost & Sullivan

Frost & Sullivan, the Growth Partnership Company, partners with clients to accelerate their growth. The company's TEAM Research, Growth Consulting and Growth Team Membership empower clients to create a growth-focused culture that generates, evaluates and implements effective growth strategies. Frost & Sullivan employs over 45 years of experience in partnering with Global 1000 companies, emerging businesses and the investment community from more than 30 offices on six continents. For more information about Frost & Sullivan's Growth Partnerships, visit <http://www.frost.com>.

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