Developing Therapies for Diseases of Misfolded Proteins

BioPartnering Conference
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11 Deer Park Drive, Suite 103
Monmouth Junction, New Jersey 08852
www.exsar.com
ExSAR Corporation is a small, privately-owned biotechnology company that was founded in 1997, incorporated in Delaware, and has a current headcount of 7 employees all based in New Jersey.

ExSAR is best known for our proprietary mass spectrometry-based platform technology known as Hydrogen/Deuterium Exchange (aka H/D-Exchange), which we offer to clients on a fee-for-service basis.

The Company leveraged its H/D-Exchange platform to discover and develop drugs to treat protein folding disorders in 2005.

ExSAR has identified 2 small molecule compounds to treat diseases of misfolded proteins, specifically Gaucher disease and Tay-Sachs disease. It is worth noting that Tay-Sachs disease currently has **NO approved therapy or treatment available**.
EXSAR’S THERAPEUTIC TARGETS OF INTEREST

Many Neurodegenerative, Metabolic and Inflammatory Diseases Caused by Misfolding, Degradation and/or Aggregation of Proteins

- Combined market >$15 billion, e.g. Parkinson, ALS, Diabetes
- Our therapeutic area of interest is in diseases caused by misfolded proteins, and more specifically a smaller subset known as Lysosomal Storage Disorders (LSDs)
- Common LSDs include Fabry, Gaucher, Pompe, Tay-Sachs, and Sandhoff Diseases
- Both Tay-Sachs and Gaucher are classified as Orphan diseases as each affect a very small group of individuals. Tay-Sachs & Sandhoff (which is closely related) have a disease prevalence of ~1,500 patients worldwide; Gaucher, the most common LSD, has a disease prevalence of ~11,000 patients worldwide
- Recent studies have shown a potential link between Gaucher disease and Parkinson’s disease
ExSAR’s key value drivers

- Currently no effective therapy or treatment exists for Tay Sachs disease, so there is no competition in this space.

- There is competition in the Gaucher disease area comprised of a handful of companies, most notably is Genzyme Corporation - with their enzyme replacement therapy (ERT) drug Cerezyme®.

- However, the mechanism of action of EXR-202 is notably different and acts as a pharmacological chaperone to help in the protein refolding that is required.

- With regards to Cerezyme®, due to the high costs associated with using this drug (~$200K per patient/per year for life), a small orally-delivered pharmaceutical chaperone, such as EXR-202, would be a cost effective and hence attractive alternative to ERT, which is the current standard of care.

- ExSAR currently has 3 patents pending for the use of these compounds in their respective markets.
Developed Relationships with Leading Institutions

- **Tay-Sachs Disease and Gaucher Disease**
  - Source of ExSAR’s license for EXR-101 and EXR-202 and site of preclinical work completed by Dr. Don Mahuran of Hospital for Sick Children, Toronto
  - ExSAR helped fund a small Open Label Phase I/II of EXR-101 on Tay-Sachs completed by Dr. Joe Clarke at Hospital for Sick Children in Toronto in 2010
  - ExSAR sponsored a small Phase I/II trial of EXR-202 on Gaucher completed by Dr. Ari Zimran at Shaare Zedek Medical Center in Israel in 2010

- **Familial Amyotrophic Lateral Sclerosis (ALS)**
  - Collaborative research agreement with Dr. Jeffrey Agar of Brandeis University to identify and validate inhibitors of superoxide dismutase (SOD1) aggregation
## KEY PERSONNEL

### Experienced Leadership Team

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Companies/Institutes</th>
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<tbody>
<tr>
<td>Robert F. Johnston</td>
<td>President &amp; CEO</td>
<td>Pharmos, Sepracor, Cytogen, I-STAT, Genex, Envirogen</td>
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<tr>
<td>Charles Cantor, PhD</td>
<td>Board Member</td>
<td>CSO &amp; Chairman, Sequenom</td>
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<tr>
<td>Myra Williams, PhD</td>
<td>Board Member</td>
<td>former CEO Molecular Applications Group</td>
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<td>F. Raymond Salemme, PhD</td>
<td>Board Member</td>
<td>CEO, Redpoint Bio &amp; founder 3D Pharma</td>
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<tr>
<td>Robert Towarnicki</td>
<td>Board Member</td>
<td>President &amp; CEO, Makefield Therapeutics</td>
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### Scientific Advisory Board

<table>
<thead>
<tr>
<th>Name</th>
<th>Institution</th>
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<tbody>
<tr>
<td>William DeGrado, PhD</td>
<td>University of Pennsylvania</td>
</tr>
<tr>
<td>Edwin H. Kolodny, MD</td>
<td>NYU School of Medicine</td>
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<td>Arnold Levine, PhD</td>
<td>Institute for Advanced Study</td>
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<td>Walter Englelander, PhD</td>
<td>University of Pennsylvania</td>
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<tr>
<td>Patrick Griffin, PhD</td>
<td>The Scripps Research Institute</td>
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Worldwide Prevalence of Gaucher Patients is Approximately 11,000

- ExSAR anticipates charging a price of $60,000
- Annual peak sales are estimated to be $100 - $130 million

Potential Annual Revenue Based on Market Penetration

![Graph showing potential annual revenue based on market penetration.](image)
EXR-101: TAY SACHS OPPORTUNITY

Worldwide Prevalence of LOTS and Sandhoff Patients is Approximately 1,500

- ExSAR anticipates charging a price of $60,000 representing a $90 million market opportunity worldwide
- Annual peak sales are estimated to be $30 - $40 million

Potential Annual Revenue Based on Market Penetration

![Graph showing potential annual revenue based on market penetration](image-url)
## REVENUE PROJECTIONS

*Figures based on annual therapy costs of $60K for each drug*

### EXR-202

<table>
<thead>
<tr>
<th>Year</th>
<th>Gaucher (WW)</th>
<th>% Registered</th>
<th>% Penetration</th>
<th>Patients on ExSAR-202</th>
<th>Annual Therapy Cost ($)</th>
<th>Annual Revenue ($)</th>
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<tbody>
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<td>1</td>
<td>11,000</td>
<td>70%</td>
<td>1%</td>
<td>77</td>
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<td>7%</td>
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<td>12,145</td>
<td>75%</td>
<td>18%</td>
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### EXR-101

<table>
<thead>
<tr>
<th>Year</th>
<th>LOTS/SD Prevalence (WW)</th>
<th>% Registered</th>
<th>% Penetration</th>
<th>Patients on ExSAR-101</th>
<th>Annual Therapy Cost ($)</th>
<th>Annual Revenue ($)</th>
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<td>50%</td>
<td>20%</td>
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<td>55%</td>
<td>55%</td>
<td>501</td>
<td>60,000</td>
<td>30,058,600</td>
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FUNDING MILESTONES

$15 Million Funding

EXR-101

- IND
- P1
- P2

H1 ‘12 H2 ‘12 H1 ‘13 H2 ‘13 H1 ‘14 H2 ‘14

$5.2 Million

EXR-202

- IND
- P1
- P2

$5.1 Million

G&A/Research/Service Business Expenses

$1.9 Million
FUNDING

- Raised approximately $12 million to date, with $11 million coming directly from ExSAR’s President and CEO, Robert Johnston.

- Received Grant from FDA (DHHS) for Clinical Studies of Safety and Effectiveness of Orphan Products for $400K in 2008

- ExSAR is seeking $15 million, which will enable us to initiate and complete more extensive Phase I/II clinical trials, and to identify and in-license additional compounds.

- We will be looking for corporate partnerships to conduct the pivotal Phase III trials and commercialization.
ExSAR is supported by a strong, experienced leadership team of executives, directors and scientific advisors.

ExSAR has 2 small molecule compounds: EXR-101 and EXR 202, both targeting orphan indications, one of which currently has NO effective treatment or therapy.

Both compounds have a well-established safety profile in humans for other indications, and each is likely to provide 7 to 10 years of market exclusivity in both the US and Europe respectively, upon being granted Orphan Designation by the regulatory agencies. ExSAR has already filed an IND for EXR-101.

We are asking for $15 million in funding to support additional clinical trials of both EXR-101 and EXR-202.

ExSAR is interested in exploring corporate partnerships in order to conduct Phase III trials and commercialization of these drugs.
Thanks to & thank you all for your attention!

Robert F. Johnston  President & CEO  rjohnston@exsar.com

Dawne Miller  Head, Business Development  dmiller@exsar.com

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