ExSAR™

Developing Therapies for Diseases of Misfolded Proteins

BioPartnering Conference
May 23-24, 2011

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Monmouth Junction, New Jersey 08852
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ExSAR INTRODUCTION

Corporate Snapshot

- **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**

COMPETITION & INTELLECTUAL PROPERTY

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.

BUILDING A PIPELINE THROUGH COLLABORATIONS

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 superdioxide dismutase (SOD1) aggregation

KEY PERSONNEL

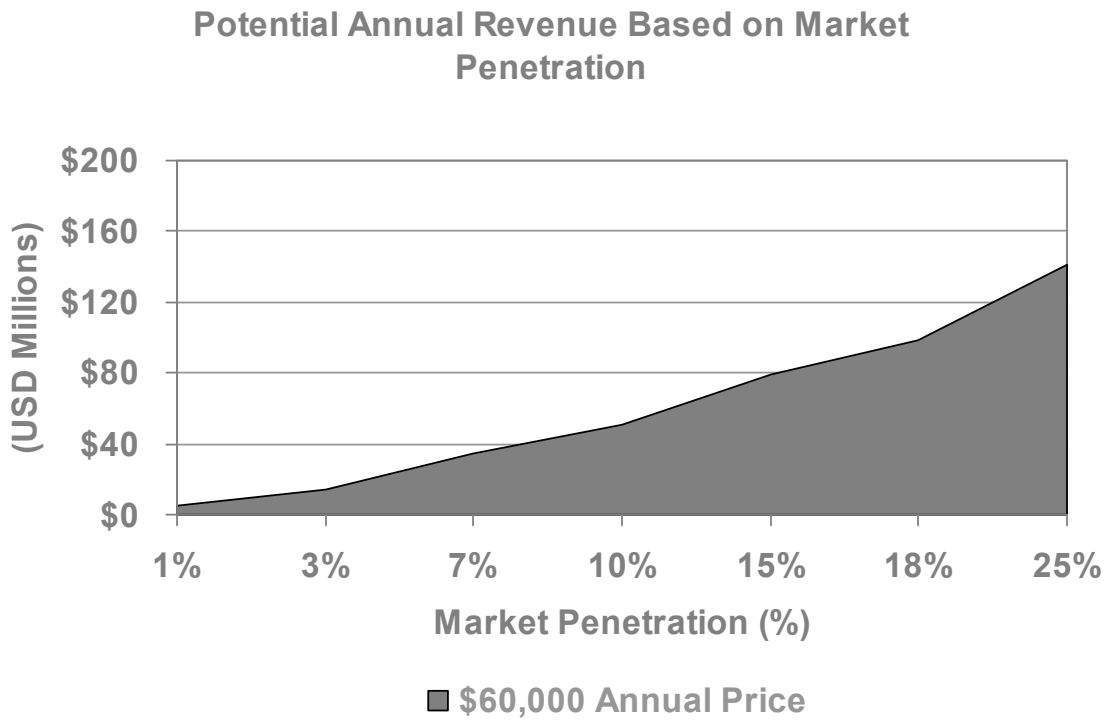
Experienced Leadership Team		
Robert F. Johnston	***President & CEO***	***Pharmos, Sepracor, Cytogen, I-STAT, Genex , Envirogen***
Charles Cantor, PhD	***Board Member***	***CSO & Chairman, Sequenom***
Myra Williams, PhD	***Board Member***	***former CEO Molecular Applications Group***
F. Raymond Salemme, PhD	***Board Member***	***CEO, Redpoint Bio & founder 3D Pharma***
Robert Towarnicki	***Board Member***	***President & CEO, Makefield Therapeutics***

Scientific Advisory Board	
William DeGrado, PhD	***University of Pennsylvania***
Edwin H. Kolodny, MD	***NYU School of Medicine***
Arnold Levine, PhD	***Institute for Advanced Study***
Walter Englander, PhD	***University of Pennsylvania***
Patrick Griffin, PhD	***The Scripps Research Institute***

EXR-202: GAUCHER OPPORTUNITY

Worldwide Prevalence of Gaucher Patients is Approximately 11,000

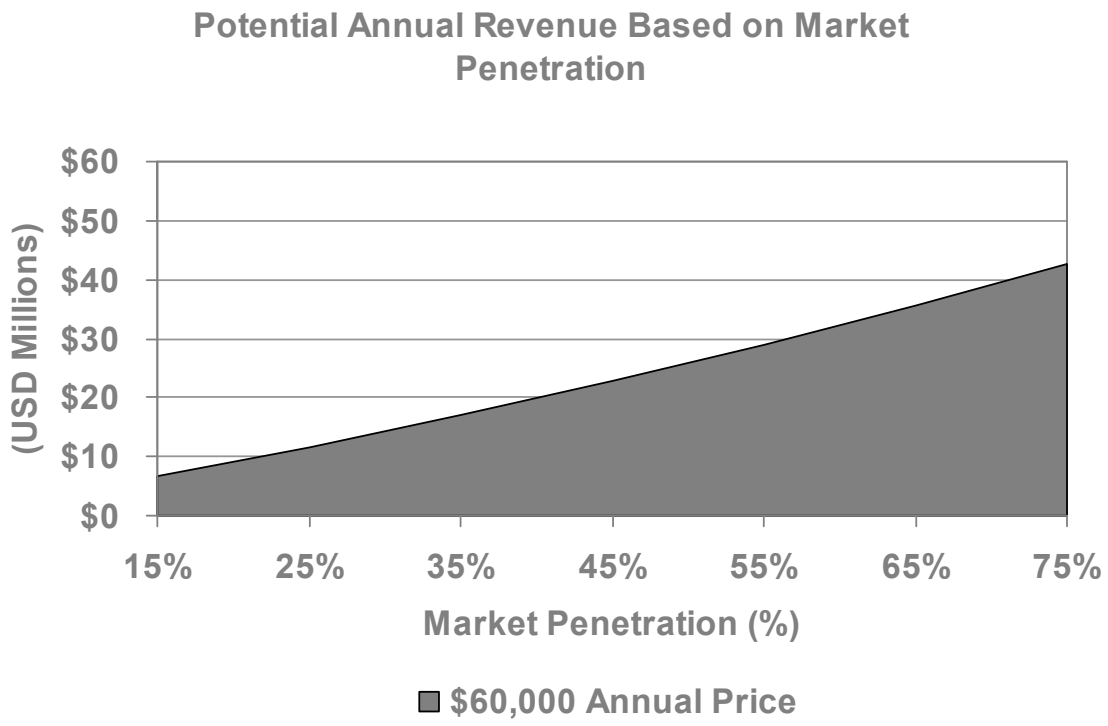
- ExSAR anticipates charging a price of $60,000
- Annual peak sales are estimated to be $100 - $130 million



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**



REVENUE PROJECTIONS

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

EXR-202

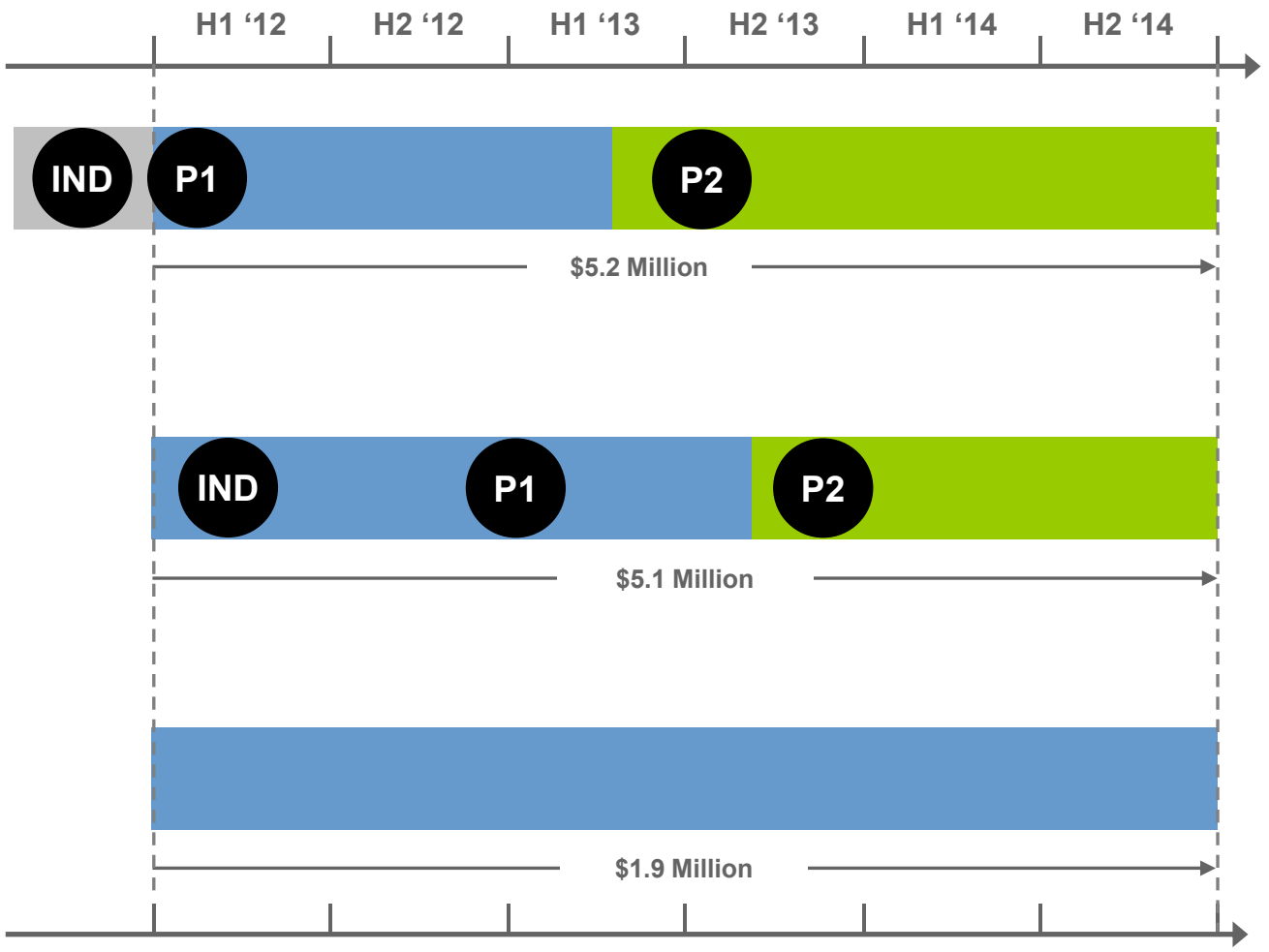
	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Gaucher (WW)	11,000	11,220	11,444	11,673	11,907	12,145
% Registered	70%	71%	72%	73%	74%	75%
% Penetration	1%	3%	7%	10%	15%	18%
Patients on ExSAR-202	77	239	577	852	1,322	1,640
Annual Therapy Cost ($)	60,000	60,000	60,000	60,000	60,000	60,000
Annual Revenue ($)	4,620,000	14,339,160	34,607,866	51,129,001	79,298,980	98,373,600

EXR-101

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
LOTS/SD Prevalence (WW)	1,500	1,530	1,561	1,592	1,624	1,656
% Registered	50%	51%	52%	53%	54%	55%
% Penetration	20%	30%	40%	50%	53%	55%
Patients on ExSAR-101	150	234	325	422	460	501
Annual Therapy Cost ($)	60,000	60,000	60,000	60,000	60,000	60,000
Annual Revenue ($)	9,000,000	14,045,400	19,476,288	25,309,811	27,618,257	30,058,600

FUNDING MILESTONES

$15 Million Funding



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.

SUMMARY

- 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.

ExSAR

Thanks to BioNJ & thank you all for your attention!

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