A Balanced, Value-creating Biotechnology Model with 6 Therapeutic Platforms

1. REMODULIN® (treprostinil) Injection
2. TYVASO® (treprostinil) Inhalation Solution
3. orenitram® treprostinil Extended-Release Tablets
4. NCE/Novel Biologics\(^{(1)}\)
5. Unituxin® (dinutuximab) Injection
6. Organ Manufacturing

\(^{(1)}\) NCE = New Chemical Entity
REMUDULIN® THERAPEUTIC PLATFORM

NEXT-GENeration Enabling Technologies for Remodulin®

IMPLANTABLE SYSTEM FOR REMODULIN® (ISR)

REMUUNITY™

TREVYENT®[1]

FDA APPROVED

FDA CLEARED

Remodulin® subcutaneous pump refills: SIMPLIFIED

The world’s first pre-filled, pre-programmed infusion system containing treprostinil

New & Innovative SMART PUMP TECHNOLOGY

[1] Trevyent® is a development-stage product not approved for sale in any jurisdiction.
TYVASO® THERAPEUTIC PLATFORM

NEXT-GENeration Enabling Clinical Trials and Technologies for Tyvaso®

INCREASE
Phase III clinical trial in PH WHO Group 3

TYVASO® for PH ILD\(^{(1,2)}\)

PERFECT
Phase III clinical trial in PH WHO Group 3

TYVASO® for PH COPD\(^{(1,3)}\)

TREPROSTINIL TECHNOSPHERE\(^{(4)}\)
Oral inhalation technology in PH WHO Group 1

\(^{(1)}\) Tyvaso® is not approved for PH WHO Group 3 patients. \(^{(2)}\) PH ILD = Pulmonary Hypertension associated Interstitial Lung Disease. \(^{(3)}\) PH COPD = Pulmonary Hypertension associated with Chronic Obstructive Pulmonary Disease. \(^{(4)}\) Treprostinil Technosphere® is a development-stage product not approved for sale in any jurisdiction.
ORENITRAM® THERAPEUTIC PLATFORM

NEXT-GENeration Enabling Clinical Trials for Orenitram®

FREEDOM-EV
Combination therapy in PH WHO Group 1

Meets primary endpoint with a 25% decrease in the risk of morbidity/mortality event¹

FDA APPROVED²

TAO
Formulation optimization in PH WHO Group 1

Based on pharmacogenetic testing

¹ PAH background therapy decreased the risk of a morbidity/mortality event versus placebo by 25% (p=0.039). ² FDA approved a supplement to the New Drug Application for Orenitram® reflecting data from the FREEDOM-EV study.
## NEW CHEMICAL ENTITIES (NCE) & NOVEL BIOLOGICS

### Therapeutic Platform

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<th><strong>SM04646</strong>&lt;sup&gt;(1)&lt;/sup&gt;</th>
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<td>Phase III gene therapy clinical trial in PH WHO Group 1</td>
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**RALINEPAG**
- Next-generation, oral, selective and potent prostacyclin receptor agonist

**SAPPHIRE**
- Rebuilding lung blood vessels destroyed by PAH

**SM04646**
- A small molecule WNT inhibitor to treat IPF

**UNEXISOME™**
- A novel biologic for the treatment of BPD

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<sup>(1)</sup> Ralinepag, the Sapphire gene therapy product, SM04646 and Unexisome™ are development-stage products not approved for sale in any jurisdiction.

<sup>(2)</sup> IPF = Idiopathic Pulmonary Fibrosis. (3) BPD = Bronchopulmonary Dysplasia.
**DINUTUXIMAB THERAPEUTIC PLATFORM**

**DISTINCT**
Clinical trial for small cell lung cancer

**100% ENROLLED**

**NEXT-GENeration Dinutuximab Programs**

**DISTINCT**

**DINUTUXIMAB**
Humanized dinutuximab\(^{(1)}\) for the following diseases:

- Neuroblastoma
- Soft tissue sarcoma
- Glioblastoma
- Melanoma

\(^{(1)}\) Humanized dinutuximab is not an approved product in any jurisdiction.
ORGAN MANUFACTURING THERAPEUTIC PLATFORM

Transform the Marketplace to Make More Organs Available for Transplant

~1.0 MILLION PEOPLE IN THE U.S. who have end-stage organ disease and may need a heart, kidney or lung transplant

» EX-VIVO Lung Perfusion (EVLP)
» XENO Transplantation
» 3D Organ Scaffold Printing
» Regenerative Medicine