

Medical affairs expertise from medical strategy to scientific content

ADDITIONAL OFFERINGS

STRATEGIC MEDICAL PLANNING

SCIENTIFIC PLATFORMS

Publication planning and execution

Communication plan development

Evidence generation strategy and life cycle management

Medical expert engagement and insights

Advisory boards

MSL training

MSL slide decks & materials

Congress coverage

Competitive readiness

Congress symposia

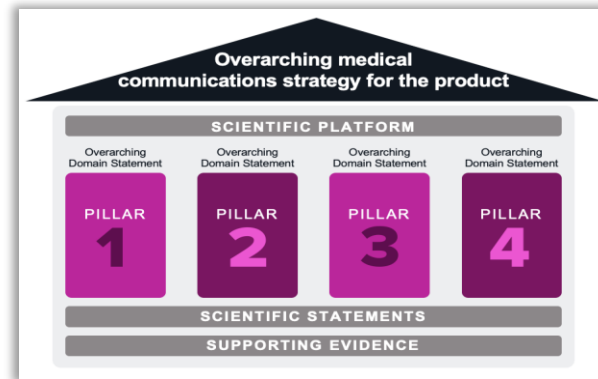
Medical booths

USMA: Medical Strategic Imperatives

USMA: MSIs and Medical Strategic Objectives

USMA: MSIs and Medical Strategic Objectives (MSOs)

1 Paradigm Shift	2 Trusted Leader	3 Treatment of Choice	4 Access	5 Long-term Opportunities
1.1 Unmet Need: Increase awareness of the SMA, correct medical need including the importance of early diagnosis.	2.1 Scientific Excellence: Generate and communicate scientifically sound evidence.	3.1 Label Support: Develop clinical and real-world data to support the safe and optimal use of ZINCYSRAN.	4.1 Value: Evaluate and communicate the value of ZINCYSRAN to enhance patient outcomes.	5.1 Internal Landscape: Evaluate and create medical strategy to support successful IT search.
1.2 Gene Therapy: Engage on the rationale and practical considerations for gene therapy.	2.2 Partnership: Engage with the scientific and medical community to advance the care of patients.	3.2 Switch: Communicate and support safe and effective switch to ZINCYSRAN.	4.2 MSL & Early Engagement: Engage with opinion leaders to engage general testing.	5.2 IT Launch Readiness: Ensure the necessary infrastructure for successful launch.
1.3 SMA: Establish the unique medical voice of gene replacement therapy to treat SMA.	2.3 Benefit/R: Communication of real world experience of efficacy, quality and safe use of ZINCYSRAN.	4.3 Barriers: Understand the barriers to access to treatment.	5.3 General Therapeutic Landscape: Develop strategic and tactical communication plans.	5.4 Keyop: Identify, understand, and communicate keyop and other treatment decisions and outcomes.



SCIENTIFIC COMMUNICATIONS

SCIENTIFIC CONTENT

Publication Plan (2020)

Design of a Global Phase 3, Randomized, Placebo-Controlled Trial of a Fixed-Dose Sodium Phenylbutyrate and Tauroursodiol Conformation in Amyotrophic Lateral Sclerosis

Study A35-004 (PHOENIX)

Rigorous Assessment of Off-Target Editing by CRISPR/Cas9 in VOR33, an Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia

PHOENIX

TRIAL to begin recruiting in Q3 2021

Complement Activation Is an Important Cause of Anti-AQP4 Antibody-Positive NMOSD^{1,2}

The Diverse Symptoms of CS Reflect Effects of Serotonin and Other Secretory Products of NETs on Multiple Organ Systems^{1,2}

Deficient LAL Enzyme Does Not Process CEs and TGs¹⁻³

Onco-genic Drivers Can Be Leveraged to Treat mTNBC¹⁻¹⁶

