Executive Summary:
The goal of Krouzon Pharmaceuticals is to develop targeted therapy for craniosynostosis syndromes caused by activating dominant FGFR germline mutations. These are orphan and ultra-rare pediatric skull disorders. Craniosynostosis syndromes including Crouzon, Pfeiffer, and Apert syndromes cause premature fusion of skull bones before the completion of brain growth resulting in abnormal skull and face, protruding eyeballs, visual impairment, deafness, respiratory distress, increased intracranial pressure, and mental retardation. If left untreated, craniosynostosis can cause chronic headaches, developmental delay, and neurological disorders including blindness. Current treatment requires complex invasive multiple surgeries throughout childhood until the age of 14 to remodel the skull and allow the brain to grow. The current cost for surgeries and postoperative care is about $1M per patient.

Market Opportunity / Unmet Need:
FGFR related craniosynostosis syndrome occurs to 1 in 15000 newborns. Treatment is also focused on administering the drug to existing patients to limit the surgeries still required. Providing treatment to new and existing patients will result in about 2000 patients per year.

Products/Services – Launched & Pipeline:
KRZ102: A lead small molecule inhibitor selected for Preclinical Trials
KRZ105: Backup to the lead molecule
KRZ119: Auxiliary to the backup molecule

Commercial / Technical Milestones:
   International Patent Publication Number # WO 03/076467 A1
2. 2005: Discovered novel therapeutic targets to treat Craniosynostosis.
   International Patent Publication Number # WO 2005/115363 A2
3. 2019: Designed and Synthesized small molecule inhibitors for the target.
   International Patent Publication Number # WO 2019/051469 A1

Competition:
Currently, there is no pharmacological treatment available for treating these diseases.

Market Size (Unaudited):
Global market size is ~$400 million (~2000 patients/year*$200k treatment cost/per patient).