Therapeutic Opportunity in Rare Disease

Rady Children's Institute Genomic Medicine

Rare diseases affect over 30 million patients in the US

75% of rare diseases affect children

95% of rare diseases lack an FDA approved treatment

450 new medicines in development for rare diseases

RCIGM Value Proposition

APPROVED DRUGS – ON LABEL	**Accelerated Patient Identification** *Improved patient identification leading to more overall volume for approved therapies*	**Real World Evidence to Demonstrate Product Value** *Leverage outcomes data in value-based pricing to expedite time to market*
APPROVED DRUGS – OFF LABEL	**Off-Label Studies** *Conduct research to help identify additional opportunities for existing approved products*	
PIPELINE PRODUCTS	**Driving R&D with New Targets** *Providing new genomic targets for early-stage R&D*	**Clinical Trial Referral** *Improving a company's ability to recruit for clinical trials and to conduct them in a more accurate, efficient manner*
	Natural History Studies *Interrogation of linked genome/phenome data*	**Indication Expansion Identification** *Leveraging databases to conduct further research to help identify additional opportunities for existing products*

Sources: 1. The Personalized Medicine Report. Personalized Medicine Coalition. 2017. 2. Accelerating Rare Disorder Patient Identification to Drive Orphan Drug Adoption. PerkinElmer. 3. Orphan Drug Sales to Reach $262 Billion by 2024. RDMAG. 5. Market Analysis Rare Disease. Journal of Immune Disorders and Therapy