


# RARE DISEASE CLINICAL TRIALS

## FACTS

THERE ARE APPROXIMATELY **7,000 RARE DISEASES** 

ONLY OF RARE DISEASES HAVE **5% FDA APPROVED TREATMENTS** 

 **50% AFFECTED ARE CHILDREN**

**CHILDREN WITH A RARE DISEASE 30% WON'T LIVE TO SEE THEIR 5TH BIRTHDAY** 

\*Source: globalgenes.org February 2019

## PATIENT RECRUITMENT AND RETENTION CHALLENGES

- 1** Small patient pool, requiring high percentage of patients with the disease to be enrolled into the trial.
- 2** Patients are often not located near investigator sites, resulting in frequent long distance travel or lodging.
- 3** Pediatric trials pose unique challenges such as social/family dynamics, emotional barriers, and practical issues including transportation, drug administration, and scheduling.

## HOME CLINICAL TRIAL VISITS ARE THE SOLUTION

They ensure all patients, regardless of geographic location or access to appropriate travel, can participate in safe and secure clinical trials.

## MRN EXPERIENCE

OVER **1,600 RARE DISEASE PATIENTS TREATED**

OVER **36,000 HOME CLINICAL TRIAL VISITS PERFORMED**

### TOP 5 INDICATIONS WORKED ON

BLOOD

RESPIRATORY

LYSOSOMAL STORAGE & METABOLIC DISORDERS

NEUROLOGY

MUSCULOSKELETAL

\*MRN Data February 2019

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