

# Spinal Muscular Atrophy

## PHASE 2

This study evaluated an investigational therapy for spinal muscular atrophy (SMA). To be eligible, participants had to be genetically diagnosed with SMA, presymptomatic, and  $\leq 6$  weeks of age. As time was of the essence, the sponsor wanted to implement a comprehensive travel and reimbursement program that would eliminate the logistical and financial concerns that could prevent a family from participating in the study.

22 MONTHS

19 PATIENTS

17 SITES

# Spinal Muscular Atrophy (SMA)

A diagnosis of SMA can be overwhelming, especially when it's for your newborn or unborn baby. Speak with us to learn about this clinical study. It is evaluating an investigational drug to see if it can prevent or delay the onset of SMA symptoms in newborns.

### COUNTRIES

7



### CHALLENGES



Randomization needed to occur within six weeks of birth and before the newborn became symptomatic – therefore educating the physician community about the study was critical.



Logistical and financial barriers had to be eliminated in order to ensure all potentially eligible patients could be considered for participation – and, if enrolled, could participate for the duration of the study.



Complex travel support had to be managed (e.g., obtaining travel visas, facilitating cross-border travel and accelerated booking) during an emotionally difficult time for parents.

### KEY TACTICS



RSG® Arrive



RSG® Card



BIO Notifier®



Referring  
Physician  
Website



Physician Emails &  
Phone Calls

The study leveraged RSG® Arrive as an adaptive solution that could fit the needs of all travelers.

Over the course of the study, there were several instances where the program evolved to meet the needs of this unique patient population. Some instances called for a patient ambassador to assist with medical care and monitoring, translation, and transportation. Other instances called for special overnight accommodations outside of a traditional hotel stay. In reducing the time it might take sites to do this coordination, site staff were freed up to focus on patient care.

### TACTIC PERFORMANCE

# 19

PATIENTS SUPPORTED

#### RSG® ARRIVE

RSG® Arrive was the key tactic that facilitated the enrollment and engagement of the 19 patients needed, and help to achieve enrollment eight months ahead of schedule.

# 193

ITINERARIES CREATED

#### RSG® ARRIVE

RSG® Arrive specialists removed major barriers to participation by arranging full service transportation (e.g., air, car), hotel accommodations, visa assistance, translation, and much more.

# 772

REIMBURSED VISITS

#### RSG® CARD

Over the course of the study, the site staff reimbursed families for 772 visits - including initial screening visits - contributing to overall study satisfaction. All families supported with RSG® Card remained in the study for the duration.

# 8

REFERRALS FROM PHYSICIANS

#### PHYSICIAN OUTREACH

BBK's referring physician program was able to generate eight patient referrals – a significant number considering the challenging population – two of which eventually consented.

