

## **Rare disease - Focus on the patient, follow the science** <sup>[1]</sup>

### Discovery <sup>[2]</sup>

There is currently no approved treatment for sIBM patients who commonly become wheelchair bound within 15 years of developing the disease.

### Video of Profiles of Strength: Julie and Charlie, Living with sIBM

In addition to helping patients, insights into rare disorders can provide scientists with a clear understanding of disease mechanisms that can prove useful to treat more common disorders. Rare disease presents a major health problem – there are about 7,000 rare diseases, affecting more than 300 million people worldwide. We thank Mr. Cleaves for sharing his experience.

### **Tags:**

Novartis Institutes for BioMedical Research <sup>[3]</sup>

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