

Challenges with Rare Disease



Diagnosis remains an issue

Available information is inadequate

HCPs often lack the skills to make an accurate diagnosis

Time to diagnosis can be >5 years



Lack of awareness

Patients and their caregivers are the experts and must function as their own advocates

Only they know what resonates with them – Patient Ambassadors teaching the company

Its as much about the patient as it is about the carer that is there to support

And boy do they have passion!



Financial Challenges/ Insurance problems

Engaging payors early

Communicating the value of the treatment is critical

Will costs of orphan drugs be justifiable or sustainable in the future?