

KEY ISSUES FOR ATMPs FOR RDS UNDER DEVELOPMENT

- **STRENGTH:** Great progress on addressing life threatening, severely disabling RD.
- **WEAKNESS:** Major challenges to proof safety and efficacy:
 - Limited evidence
 - High uncertainties on disease evolution after DMT (specially when life threatening)
 - Limited existence of validated & patient relevant endpoints.
- **RISK:** fail to identify & detect clinically relevant change as early as possible and therefore to reach patients (in a timely manner):
- **OPPORTUNITIES**
 - Complement traditional tools with others: RWE, PED.
 - Enhanced and early engagement with registry holders, key clinicians and pat reps.
 - Elucidation of a way to accommodate new findings once CT has started
- ATMPs & the CAT are probably the ideal arena to deal with these pioneering tools.