excelya

Case Study

Type:	**Geography:**	**Study Type:**	**Client:**
Rare disease, pediatric	Europe	Phase IV	Biotech

PROJECT TITLE:

"Observational Post-AMM Study (PASS: Post Authorization Safety Study) for evaluating *******® safety in children under 12 years treated for Primary ImmunoDeficiency (PID) or for Immune Thrombocytopenic Purpura (ITP)"

Objective 1

Post-authorization safety study (PASS) designed to evaluate the use of *******® in common medical practice in children under 12 years with Primary ImmunoDeficiency (PID) or with Immune Thrombocytopenic Purpura (ITP).

Study Challenges:

1) The nuances of working with Paediatric patients.

2) Very rare disease, few patients in eligible population.

3) Exhaustivity of available patient population.

Excelya Solutions:

1) We delivered staff with expertise in handling nuances of Pediatric patient studies and use of pediatric reference site in management.

2) Expert rare disease study management with high competence of staff (average study CRA experience is 5 years).

3) Study was proposed to all eligible parents/patients. Reasons for non-enrollment was collected in a register with the support of the hospital pharmacist.

Key metrics

96% Patient retention across 2 cohorts, **100%** for PID cohort

7 Specialized sites

59 patients

Study success:

Excelya delivered study recruitment goals for very rare disease on time, very high patient retention.