QED Rare Indication Case Study

“A better partnership means better patient access”

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Case Study – Project Delivery (1/8)

Rescue of a global phase II study in an orphan haematological indication

Study objective

An international, phase II, exploratory dose-finding study with cohort design, in an orphan disease indication

Study design

Safety & efficacy of a single dose (IV injection, changed to IV infusion with amendment) medication in various dose levels and evaluation the appropriate dose range for future Phase III

Challenges

- Rare disease, with recombinant product
- Dissatisfied Sponsor
- Demotivated sites
- Exhausted patient pool
- Quality concerns
- IMP issues led to recruitment put on hold, introduction of Amendment, change in mode of administration

Case Study – Project Delivery (2/8)

Rescue of a global phase II study in an orphan haematological indication

- Efficient handling of hand-over activities.
- Re-assessment of existing sites: motivation, patient population, training on amendment of new IMP administration
- Identification, selection and set up of additional countries and sites with expertise and high recruitment potential
- Coordination of Amendment submission and approval in existing countries (change in IMP administration)
- Management of recruitment for remaining cohorts and completion of LPI on schedule

Case Study – Project Delivery (3/8)

Rescue of a global phase II study in an orphan haematological indication

- QED implemented risk mitigation and recruitment plans to maximize recruitment efficiency and achieved study milestones within the agreed timelines.
- QED implemented a cohort management plan to minimize time between cohorts and overall study timelines.
- QED added strong local and central expertise to ensure successful management of regulatory, import and export issues in the challenging countries.
- As a result, QED managed to provide overall cost savings.

Case Study – Project Delivery (4/8)

Rescue of a global phase II study in an orphan haematological indication

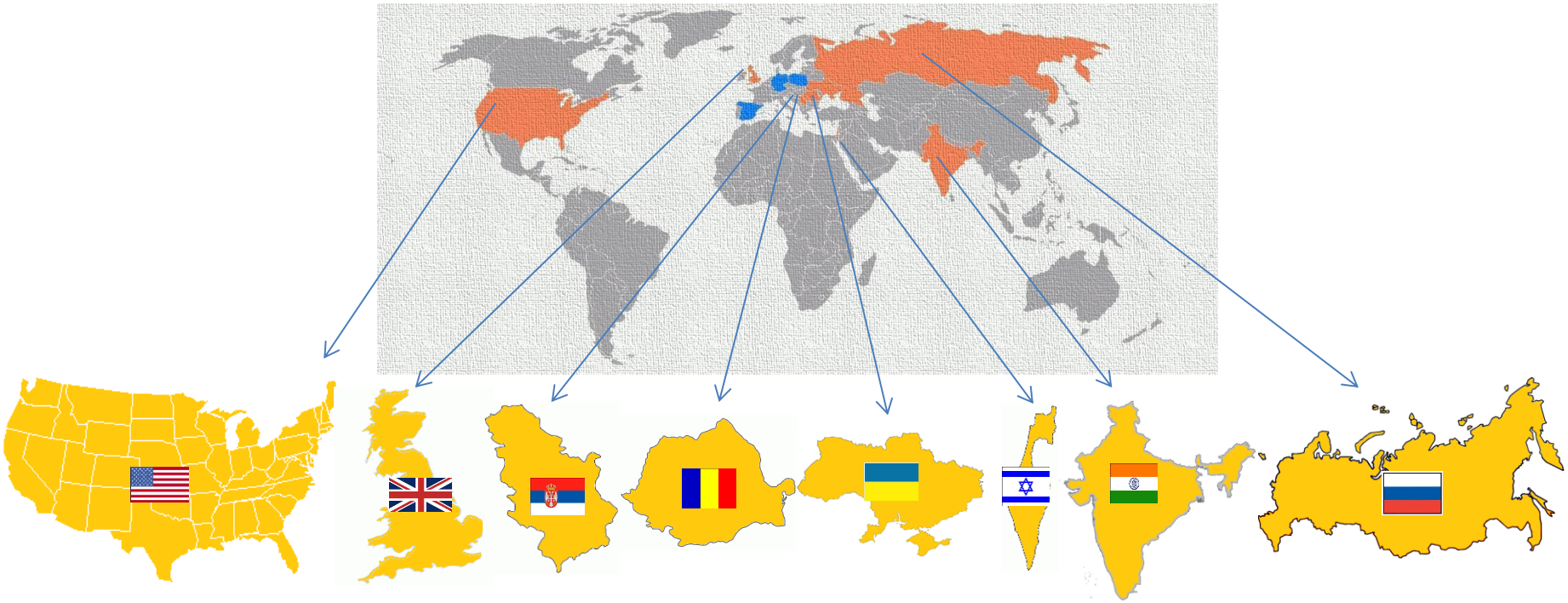
QED conducted feasibility across 5 continents, in the following countries:

- Australia
- Belgium
- Bosnia Herzegovina
- Bulgaria
- Canada
- Croatia
- Czech Republic
- Dubai
- France
- Germany
- Hong Kong
- India
- Israel
- Jordan
- Lebanon
- Macedonia
- Netherlands
- Poland
- Serbia
- Romania
- South Africa
- Spain
- Turkey
- UK
- Russia
- Ukraine
- USA

Case Study – Project Delivery (5/8)

Rescue of a global phase II study in an orphan haematological indication

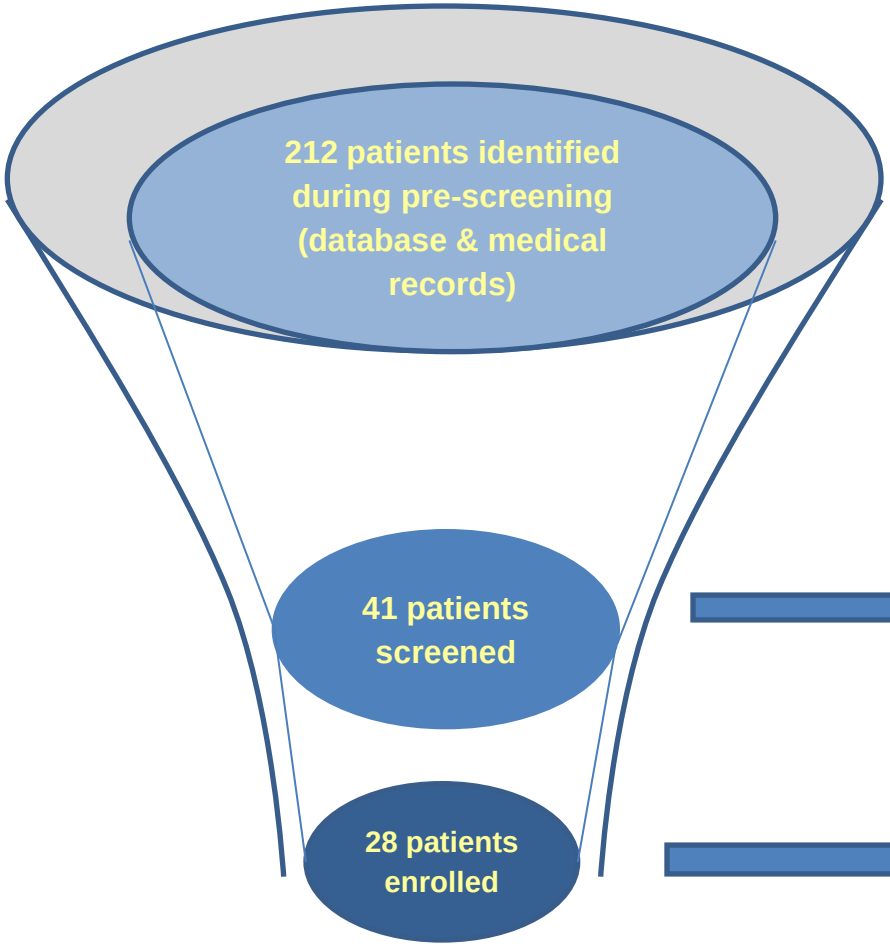
Identification and selection of 8 additional countries with strong local expertise and motivated sites with good patient recruitment potential



USA UK SERBIA ROMANIA UKRAINE ISRAEL INDIA RUSSIA

Case Study – Project Delivery (6/8)

Rescue of a global phase II study in an orphan haematological indication



Post-amendment, countries and sites selected by QED identified a total of 212 potentially eligible patients during pre-screening from their database and medical records

41 of the pre-screened patients were screened

28 were enrolled

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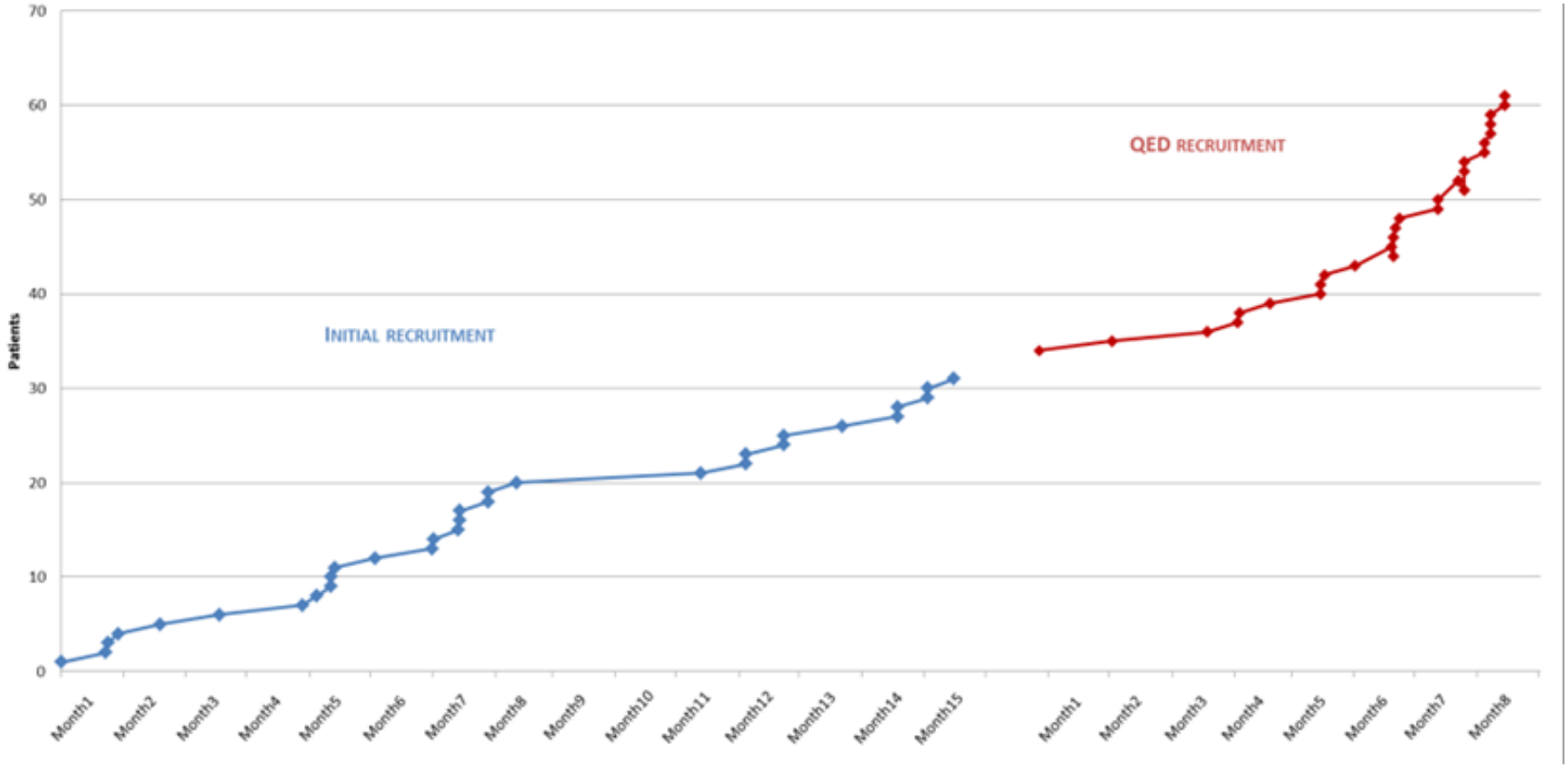
Case Study – Project Delivery (7/8)

Rescue of a global phase II study in an orphan haematological indication



Post-approval of the amendment, completion of the remaining cohorts and enrolment of the remaining 28 patients in 8 months as compared to enrolment of 32 patients in 15 months under the management of the previous CRO

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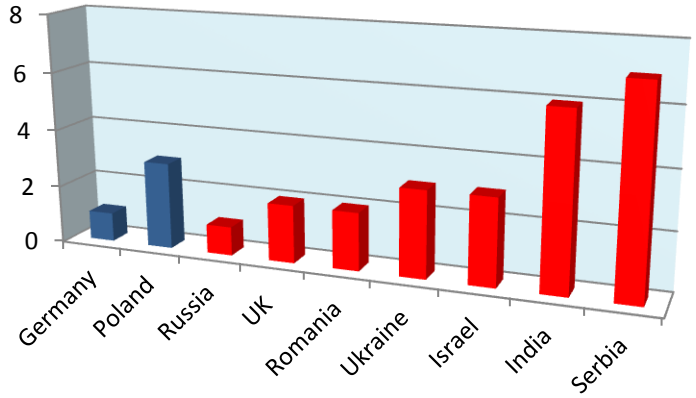
Case Study – Project Delivery (8/8)

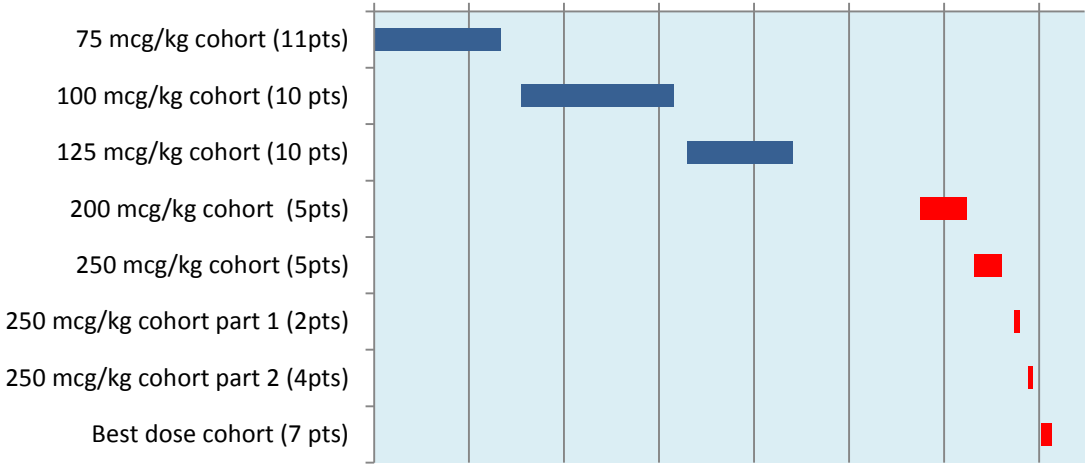
Rescue of a global phase II study in an orphan haematological indication



Of the 28 patients enrolled:

4 patients were recruited from the original sites

24 from the new sites selected by QED



By successful implementation of risk/mitigation and cohort management plans, the time between “cohort open” and “cohort closed” (i.e. all patients enrolled in the given cohort) has significantly decreased when study managed by QED compared to the previous CRO