



CASE STUDY

IDDI's Organization And Tools Overcome Trial Design Challenges In A Phase I/II Myelodysplastic Syndrome Study

Study Description

A phase I/II open-label, multi-center, dose-finding, safety, efficacy study on patients with higher risk myelodysplastic syndrome (MDS) and untreated acute myeloid leukemia (AML) who are ineligible for induction chemotherapy.

The phase I part of the study consisted of a dose exploration administered in 18 patients at a starting dose once every two weeks in a 28-day cycle. The dose was escalated and evaluated for the occurrence of dose limiting toxicities using a standard 3+3 dose escalation design. This dose exploration phase of the study established safety, tolerability, and the recommended phase II dose and schedule in adult patients with higher risk MDS.

The phase II part enrolled 175 patients and employed an open-label 2:1 randomized design to evaluate the effect of treatment at the recommended phase II dose determined in phase I.



Indication

Myeloid malignancies



Patient population

193 patients

Both phases: adult patients with a documented diagnosis of relapsed/refractory MDS or higher risk MDS previously untreated. Phase II only: bone marrow with myeloblasts constraints.



Study duration

32 months



Regions

6 US centers



Situation

IDDI as the biometry partner to ensure data quality

- IDDI had been selected earlier for the review of previously collected outputs and for the biostatistics support of the sponsor's program including the generation of trial statistical designs, protocols and statistical analysis plan development. In this particularly important phase, the sponsor benefited from IDDI's rich experience in oncology, trial design, and relationship with regulatory authorities including pre-IND meeting package submission.
- The sponsor selected IDDI as the full biometric partner including eClinical tools (EDC & RTSM), data collection, data cleaning, and data analysis. It was important to have these tasks executed by one vendor to ensure data quality and coherence with trial design.
- The sponsor selected another vendor for clinical and medical monitoring, ensuring a clear separation of tasks.

IDDI tasks



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IDDI tasks



Multiple Challenges

Fast access to all collected data, including eCRF, lab data, RTSM, external systems, etc.

Complex protocol design and specific data collection requirements combined to ease site burden

- Implementation of the dose escalation algorithm in the eCRF was particularly important to the sponsor as it would determine the recommended phase II dose
- eCRF ease of use for tumor and radiological assessments – importance of consistent lesions identification throughout the study
- Drug supply management system (RTSM) to be correlated with eCRF
- Payment sheets linked to schedule visits

Covid-19 implementation

- COVID-19 could create missed visits, missed data – sponsor wanted a mechanism to ensure study goals would still be met
- Importance of risk-based monitoring – need to apply a monitoring plan directly in the EDC to direct CRAs in source data verifying the required fields



Solutions (1)

IDDI offered to deploy its fully integrated eClinical suite consisting of ID-base (EDC) and ID-net (RTSM)

Webservices allow IDDI's EDC system to communicate with other systems whether internal to IDDI (such as RTSM) or external systems.

Adhoc reporting allows users to build their own reports based on their own needs content wise and timewise.

Use of calculation functionalities to copy the target and non-target lesions documented at screening into the post treatment visits, ensuring consistency in lesion identification across the entire study, reducing data entry by sites.

Dose escalation algorithm implemented into eCRF to allow determination of phase II dose.



Solutions (2)

Clear separation between monitoring and biometry teams clinical data management (CDM), RTSM, and biostatistics teams coordination

CDM linked to biostatistics services and kept separated from monitoring, allowing two completely independent views of the data (monitors and biometry team); the link between CDM and biostatistics ensured collected data met the requirements of the study objectives.

The main element is the expertise of the clinical data manager. When it comes to eCRF design the combination of lead clinical data manager / EDC specialists is the key to success.

Key data management and biostatistics milestones integrated in the planning: SDTM part of the CDM team allows to start programming the datasets as soon as eCRF is finalized. Biostatistics team works on ADaM datasets and clear any data issues directly with the CDM team.

Seamless protocol amendment implementations: Lead CDM, biostatisticians and MD all reviewed the protocol (schedule of assessments and data to be collected).

Once the protocol was finalized and eCRF developed, the biostatisticians review ensured collected data could be easily analyzed and all endpoints were included.



Results

- Thanks to IDDI's expertise, solutions-focused and adaptable approach, coherent submission-ready clinical data were delivered.
- IDDI's strategies helped the client to not only meet the challenges of this collaborative global trial, but also foster harmonized clinical data management

**IDDI has
successfully
delivered**

- Fast implementation of a complex design thanks to integrated technology
- Easy and precise determination of phase II dose
- Coordinated identification and resolution of missing data due to Covid-19
- Coherence between SDTM and ADaM datasets





Contact Us

Whether you need assistance with your clinical strategy, study design, clinical data management, or biostatistical analysis, IDDI's experts are here to help.

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About IDDI

IDDI is a trusted clinical data science partner. With 35+ years of research-driven experience, we combine therapeutic expertise, biostatistical leadership, and regulatory insight to mitigate risk from design to registration and beyond.

We deliver uncompromising excellence in biostatistics, strategic consulting, clinical data management, IDMCs, and supporting eClinical technologies, because when every data point represents a patient, perfection is the only option.