BACKGROUND

HR MDS represents approximately 30% of all MDS cases; currently there are no approved treatments for HMA failure HR MDS and it is a broader population. This has been recognized by the FDA as an acceptable enrichment strategy for clinical trials; an increase in sample size may influence investigator interest and behavior in study participation and enrollment. In the present setting, the rationale and outcomes of the adaptive study design need to be clearly communicated to study investigators at the beginning of the study as well as following the IA.

SUMMARY

As outlined in the HR-MDS domain, all respondents agreed that an adaptive trial design with sample size re-estimation is a reasonable and acceptable approach to reduce the risk of underpowered studies. The most commonly cited benefit of adaptive trial designs was the ability to provide flexibility and adaptability to evolving scientific knowledge.

REFERENCES/ACKNOWLEDGEMENTS