Cognitive impairment associated with schizophrenia (CIAS) is linked with... and visual attention—often cover multiple domains. However, there were no appropriate CANTAB domains that could be pre-specified as the primary endpoint for Stage 2.

RESULTS

Stage 1

At the end of Stage 1 the blinding of all doses was maintained. However, in each of the active treatment arms, no CANTAB endpoints were identified that differentiated between BI 409306 and placebo with an effect size of 0.5 or greater.

In the absence of pre-specified CANTAB domains for the primary endpoint, an alternative approach was used to choose a composite score. Change from baseline in the composite score of MCCB after 12 weeks of treatment was therefore specified as the primary endpoint for Stage 2.

CONCLUSIONS

The novel and adaptive ‘Learn and Confirm’ study design allows for the identification of meaningful endpoints in Stage 1 of a trial, which can then be used for the primary endpoint in a subsequent confirmatory analysis in Stage 2. However, there were no appropriate CANTAB domains that could be pre-specified as the primary endpoint in the present study, and therefore the composite score of MCCB was selected as the primary endpoint for Stage 2. When the most relevant measure for an expected drug effect cannot be pre-specified from a range of relevant tests at the start of a study (as was the case with the multitude of cognitive domains and tests in the present study), the ‘Learn and Confirm’ approach provides a methodologically rigorous alternative.

DISCLOSURES

The authors met the criteria for authorship as recommended by the International Committee of Medical Journal Editors. Four of the authors (DK, FK, DR and PB) are employees of Boehringer Ingelheim, but received no direct compensation related to the development of this poster. DB has no disclosures to note. MD served as the consultant to receive the poster for medical and scientific accuracy as well as intellectual property considerations.

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