Challenges on Outcomes Selection Along Early to Late Phase Clinical Trials of Drug Development: Survey to Experts in Neurosciences Drug Development*

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INTRODUCTION

Despite major advances in neuroscience, clinical trials for neurological and psychiatric conditions continue to have notoriously high failure rates. The use of innovative Clinical Outcome Assessments (COAs) grounded in translational research is key to maximize the likelihood of identifying promising new treatments in early phase clinical trials. However, the field has lacked standardized practice guidelines for optimal selection of COAs and also face a low acceptance of innovative outcomes by the regulators or, eventually, health agencies.

OBJECTIVE

For the recently developed **7-step standard process**[1,2] for COA selection, we wanted to explore the acceptance of the process, and to identify challenges for its implementation by pharmaceutical and biotechnology drug developers.

7-Step Process

RESULTS

Twenty-six participants, answered affirmatively to reading the pre-survey materials, mainly from pharmaceutical industry (**46%**) and with substantial years of experience in COA strategy decisions (**80% more than 10 years**) (Figure 1). All participants endorsed the **7-Step** process, with some suggestions for additional activities summarized in Table 1. Results of text analysis are shown in Figures 2 and 3.

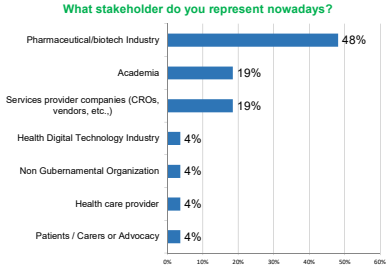

Figure 1. Stakeholder Representatives

METHODS

A survey was administered to international experts on outcome assessment research to solicit feedback on the proposed 7-step process. All participants conducted a pre-reading activity of briefing materials describing the established standard process. Open-ended questions were posed including level of agreement, endorsement and expected challenges when implementing the 7-step process. An initial qualitative analysis of the open-ended questions is presented using the software Voyant tools for text analysis.

- From your experience, to which extent is the pharmaceutical industry currently using the full 7-Step process?

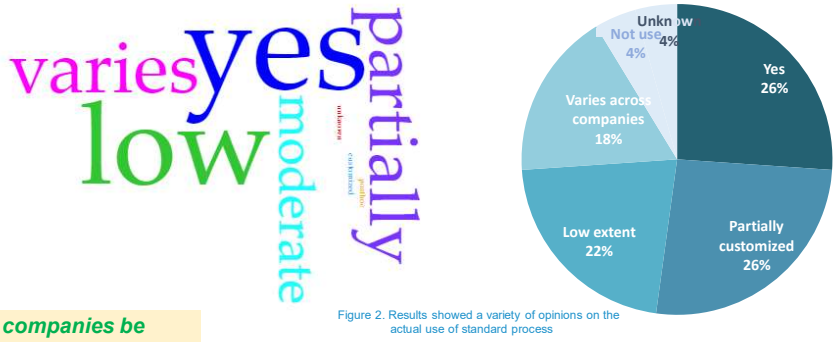

Figure 2. Results showed a variety of opinions on the actual use of standard process

- In your opinion, how can drug development companies be encouraged to adopt the proposed standard process?

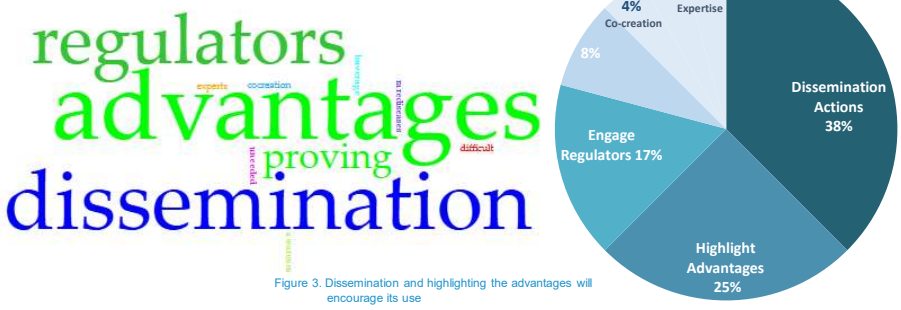

Figure 3. Dissemination and highlighting the advantages will encourage its use

Table 1. Additional Recommendations to Include in the Standard Process

- *"It is not clear to me in the stakeholder engagement if the **regulators** are involved (policy makers?) If not, I believe there should be an alignment early on". S24*
- *"I would like to include the **time need to perform the assessment, if the COS is the primary or secondary objective** in a clinical trial, and the other COSs in the trial because all of these items impact patient, caregiver, and research site staff burden which impacts the quality of data derived from the COS)." S13*
- *"Interesting also to be applied for novel **brain-computer interfaces (BCIs)** as the most outstanding or novel CNS trials." S12*
- *"I think more closely **aligning** these steps, **with regulatory guidance** (e.g., FDA COA guidance documents) would make the value of this formal step-wise approach more clear". S16*
- *"In step 6- is only mentioning drug labelling, but not sure about potential diagnostics." S11*
- *"Potentially, in Alzheimer's we published a 'heat map' to show **gaps between existing COAs and the concept elicitation items**. Also (not common) but with Alzheimer's we identified so many items that we are ranking them within health concept in a study." S07*

CONCLUSIONS

- The consensus-based 7-step process for setting COAs strategy in neuroscience clinical should be a first reference for any type of research in drug development in neurology and psychiatry.
- Feedback obtained from experts is near universally in favor of its adoption, as risk-mitigation based upon its adoption, and to facilitate the interaction with regulatory agencies to reach alignment.
- There was a wide range of opinions regarding the extent to which the industry is currently using standards, with half of the participants acknowledging the use either fully or customized at some extent.
- Several survey participants described a roadmap of future activities aiming to educate, disseminate and promote the use of the 7-step standard method for future drug development programs.
- Additional activities suggested were addressed to highlight the advantages in terms of time/cost to drug development and align the process with the existing regulatory guidance in USA (i.e. PFDD) and Europe.
- More participants are needed to increase expert representation.

Willing to take the survey? Visit the Pre-Readings material and the survey or scan the QR

Take the Survey

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