Telecon with Sponsor, July 29, 2011 - Hemacord

BLA 125397, NYBC cord blood

Date: 7/29/11

Time: 1:00 pm – ~1:20 pm ET

FDA Participating: Wilson Bryan, John Hyde

NYBC Participating:
Pablo Rubinstein, M.D. – Vice President, New York Blood Center (NYBC) and Director, National Cord Blood Program (NCBP)
Andromachi Scaradavou, M.D. – Medical Director (NCBP)
Michael Zdanowski – Director of Operations (NCBP)
Edwin W. Streun – Director, Regulatory Affairs-I (NYBC)

FDA initiated

The FDA opened by stating the purpose of the call was to follow up on the recent teleconference of 7/27. In that teleconference, the FDA had raised the issue that it was finding difficulty with relying on the Docket dataset and published information to provide the necessary substantial evidence of efficacy for certain indications, and the FDA had asked the NYBC if they could provide additional data.

The FDA said they wanted to be sure NYBC is aware that the FDA plans to raise these issues at the upcoming Advisory Committee (AC) meeting so that NYBC is adequately prepared for the meeting. The indications involved are those other than hematologic malignancies, with the exception of SCID.

The FDA said they have searched the literature but are having trouble identifying adequate control populations for most of the nonmalignant indications. The NYBC said they could provide literature. The NYBC noted that it might be possible to get some more data on the patients represented in the datasets they have already submitted, but that would be more than is usually provided to cord blood banks. The NYBC might be able to get neuropsychiatric data, but that might entail a lot more work. The FDA noted that the value of such data would depend on whether control data are available for comparison. The FDA recommended doing a preliminary assessment of what data could be obtained and how that might be able to contribute to supporting the indications, before investing heavily in obtaining the data.

The NYBC asked if this meant the cord blood guidance document would be discussed at the AC meeting. The FDA answered that the meeting would be specifically for the BLA and not about the guidance document.

The NYBC asked if there might be discussion at the AC meeting about changing the indication into an indication for hematopoietic reconstitution, or if that idea could be considered. The FDA replied that the AC meeting was to address the specific indications requested by the NYBC, which, in this case, would be those listed in the guidance, because that is what was requested in the BLA application. The FDA remarked that it saw regulatory obstacles to considering hematologic reconstitution as the indication.

The NYBC asked if the FDA could provide a list of specific data they needed. The FDA responded that they could not be much more specific at this point that what they already said.

The NYBC asked how soon they needed to submit the information, as some of it could take a while to obtain. The FDA noted that reviews would not be concluded until after AC meeting. The FDA recommend that the NYBC first do an assessment of what additional data they could obtain, what work it would take to get it, and what would be a reasonable expectation for when it could be submitted. Then there could be further discussions. The FDA noted the AC planning meeting that the NYBC requested might be scheduled for next week, and these issues could be discussed more then. The FDA recommended that the FDA and the NYBC maintain an active dialogue about how to approach the submission of additional data to support efficacy.

The NYBC asked how the information they obtained should be submitted. The FDA responded that it should be submitted as an amendment to the BLA.