RP-103 Clinical Trial Update for the European Cystinosis Community

Horizon Pharma plc is pleased to share an update regarding the availability of delayed release cysteamine bitartrate gastro-resistant hard capsules for European patients currently receiving the study drug in the RP103-04 and RP103-07 clinical trials for nephropathic cystinosis.

In recent months, Horizon has worked to implement a bridge access program with the goal of transitioning nephropathic cystinosis patients currently receiving the study drug to the commercial form of the medicine, PROCYSBI®. These steps involve working with each of the RP103 clinical trial investigators to confirm Horizon's commitment to the bridge access program, determining the investigators willingness to participate in the program, and arranging logistics which comply with local laws and regulatory guidelines. We can confirm that all investigators have been contacted and the overall response to the program has been positive. Horizon will provide an update on the bridge access program on a country-by-country basis as all steps are completed with investigators at each clinical trial site, and we expect to have final details available before the end of March. We are committed to doing everything we can so that no clinical trial patient participating in these studies will experience a gap in treatment between the end of the study and start of the bridge access program so that they will continue to benefit from treatment with delayed release cysteamine.

PROCYSBI was approved centrally by the European Commission on September 6, 2013, following a favorable benefit/risk assessment adopted by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). Since the granting of EU approval, the RP103 nephropathic cystinosis clinical trials have been extended to accommodate the time it would take for us to agree with local health authorities on the terms of market access to help all patients benefit from PROCYSBI. We are transitioning to a bridge access program because the clinical trials have met their pre-specified objectives. As Horizon explores potential options to secure appropriate local market access, we commit to the availability of the bridge access program for clinical trial patients.

The significant benefit of PROCYSBI has been strongly articulated by the Committee for Orphan Medicinal Products (COMP) of the EMA in maintaining the orphan medicinal product designation of PROCYSBI, and the outpouring of demand for continued access among study participants and their families. As a company with a long-term commitment to people living with rare diseases, including nephropathic cystinosis, Horizon is dedicated to continuing to work with national health authorities to secure fair and appropriate local market access so that all patients living with cystinosis have the option of PROCYSBI.