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For immediate release

NanoCarrier Co., Ltd  
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(Code No. 4571 Tokyo Stock Exchange Mothers Section)

## **Notice of Orphan Drug Designation of NC-6004 (biliary tract cancer) by the U.S. FDA**

NanoCarrier Co., Ltd. is pleased to announce that NC-6004 has been granted to the orphan drug designation<sup>※1</sup> from the U.S. Food and Drug Administration (FDA) for the development of a treatment for biliary tract cancer<sup>※2</sup>.

As announced, NanoCarrier is evaluating NC-6004 concurrently in three types of malignancy (non-small-cell lung cancer, biliary tract cancer and bladder cancer) in a single clinical trial of a combination therapy with NC-6004 and gemcitabine, which is a phase II study adopting a basket trial design to develop NC-6004 in the United States. Patient enrollment is expected to complete in this coming fall followed by a follow-up period and analysis, and the results will be seen in the latter half of 2018.

Please note that this announcement will have no impact on the business results for the fiscal year ending March 2018. NanoCarrier will proactively seek to obtain approval as early as possible in Japan and overseas in order to provide a new drug utilizing micellar nanotechnology of Japanese origin.

< Glossary >

<sup>※1</sup> **Orphan drug designation**

US FDA makes an orphan drug designation to promote the development of new drugs for rare diseases with 200,000 or fewer patients in the U.S. and grants 7-year exclusive first distribution rights. In addition, preferential treatment is given to obtaining subsidies from the U.S. government, tax exemptions for the cost of clinical research, waivers of fees for the new drug application to FDA, and examinations of the protocols.

<sup>※2</sup> **Biliary tract cancer**

The bile ducts function to move bile produced by the liver into the duodenum. The gallbladder is attached to the halfway point of the bile duct and collects and concentrates bile. The biliary tract consists of the bile ducts and gallbladder, and malignancies affecting the biliary tract are called biliary tract cancer. According to the U.S. National Cancer Institute, the number of patients who have newly developed biliary tract cancer in 2017 is estimated to be 40,710 (2.4 % of all cancer patients) in the United States. Biliary tract cancer is reported to occur less frequently in Western countries, while it is more common in Asian countries. Although combination therapy with gemcitabine and cisplatin is currently considered the standard therapy for biliary tract cancer, a new therapy is called for. Substituting NC-6004 for cisplatin is expected to alleviate the adverse effects and enhance the antitumor effect.

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