Evaluation of drug efficacy *in vivo* and *in vitro* using patient-derived xenograft (PDX)

Shinichiro Tsunesumi¹, Hideaki Kakinuma², Takashi Morikawa¹, Shigenori Enoki¹,
Seiichi Katayama¹, Yukiko Shimada², Hiroaki Inoue², Taisei Nomura³

¹Pharm.Dept., Kumamoto Lab., LSI Medience Corp., ²Advanced Medical Business Development, LSI Medience Corp., ³Laboratory of Animal Models for Human Diseases, Center for Rare Disease Research, NIBIOHN

To improve the success rate of anti-cancer drug development, clinically relevant tumor models are needed. Patient-derived xenograft (PDX) tumor, which is generated by direct implantation of human tumor into immunodeficient mice, has been well-accepted as a more clinically relevant model than cell line-derived xenograft (CDX) tumor.

We have been developing both *in vivo* and *in vitro* screening systems for anti-cancer drugs using PDX tumors that had been established in the National Institutes of Biomedical Innovation, Health and Nutrition (NIBIOHN). In this study, we confirmed anti-cancer drug efficacy using PDX *in vivo* and *in vitro*.

In the *in vivo* study, the PDX tumor was subcutaneously transplanted into immunodeficient mice, and a control group and drug group were set. Gemcitabine or 5-FU was administered once or twice a week, and observation was carried out until 28 days after starting administration. Anti-cancer effect was evaluated by tumor volume.

In the *in vitro* study, three-dimensional culture was performed using dispersed cells derived from PDX tumor. Drugs were added to the medium and the cell viability was confirmed by ATP assay.

In both studies, the effect of anti-cancer drug was confirmed, and an evaluation system *in vivo* and *in vitro* using PDX could be established.