SELECTED OPPORTUNITY IN ONCOLOGY

Gene therapy of pancreatic cancer

(BIO07104)

GENE THERAPY OF PANCREATIC CANCER (BIO07104)

Product factsheet

Phase I achieved

- **Combination :** gemcitabine + gene therapy products
- **Transgenes:** human somatostatin 2 receptor (sst2) + deoxycytidine kinase (DCK) + uridine monophosphate kinase (UMK) genes for preventing gemcitabine clearance by tumor cells
- **Indication:** pancreatic cancer
- **Proof of Concept :**
  - **In vitro:** in HuH7 cells (human hepatocellular carcinoma) and HepG2 (hepatoma)
  - **In vivo :** in a hamster model of pancreatic cancer (intratumoral injection)
- **Phase I achieved (pancreatic cancer)** NCT01274455
  - Non viral delivery approach (polymer)
  - No toxicity; Survival of patients : up to 22 months

"First-in-man phase 1 clinical trial of gene therapy for advanced pancreatic cancer: safety, biodistribution, and preliminary clinical findings." Buscail L et al. Mol Ther. 2015 Apr;23(4):779-89.

- **Phase II :** recruiting NCT02806687
- **Patent applications:** WO2009/056434

GENE THERAPY OF PANCREATIC CANCER

Proof of concept

Intratumoral delivery of the gene therapy product (2 plasmides/3 genes) in combination with gencitabine 8 days after implantation of tumor cells in hamster reduces tumor progression and metastasis

Table I: Tumor progression

	Tumor progression (percentage)
No DNA, No Gemcitabine	+ 800 to 1060
Gemcitabine, No DNA	+ 150 to 378
DCK:UMK:SST2	+ 200
DCK:UMK, Gemcitabine	-60
DCK:UMK:SST2, Gemcitabine	-15 to -30

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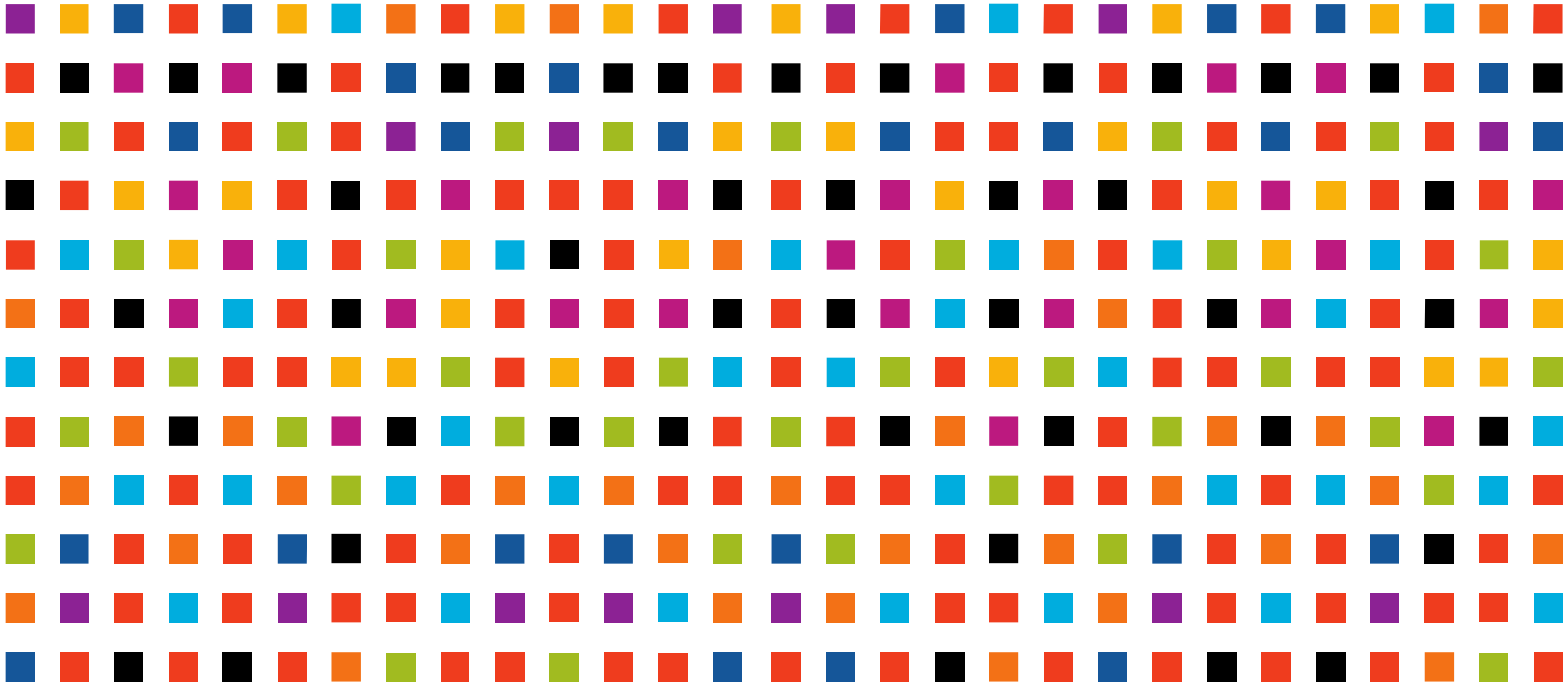
Table II: Number of metastatic sites

	Number of metastatic sites (percentage)
No DNA, No Gemcitabine (control)	100
DCK:UMK	82
DCK:UMK:SST2	100
DCK:UMK, Gemcitabine	47.6
DCK:UMK:SST2, Gemcitabine	12.2



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