

Orphan Drug Development Guidebook

Building Block I406

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	Tissue Chip for Drug Screening program and Consortium
References	https://ncats.nih.gov/tissuechip EMA Workshop on the Organ on chip use: https://www.ema.europa.eu/en/events/first-ema-workshop-non-animal-approaches-support-medicinal-product-development-challenges Additional info can be found in several publications, such as: Zhang B, Radisic M. Organ-on-a-chip devices advance to market. Lab Chip. 2017 Jul 11;17(14):2395-2420. Doi: 10.1039/c6lc01554a. PMID: 28617487. Palasantzas VEJM, Tamargo-Rubio I, Le K, Slager J, Wijmenga C, Jonkers IH, Kumar V, Fu J, Withoff S. iPSC-derived organ-on-a-chip models for personalized human genetics and pharmacogenomics studies. Trends Genet. 2023 Apr;39(4):268-284. Doi: 10.1016/j.tig.2023.01.002. Epub 2023 Feb 5. PMID: 36746737. Ma C, Peng Y, Li H, Chen W. Organ-on-a-Chip: A New Paradigm for Drug Development. Trends Pharmacol Sci. 2021 Feb;42(2):119-133. Doi: 10.1016/j.tips.2020.11.009. Epub 2020 Dec 16. PMID: 33341248; PMCID: PMC7990030.
Description	NCATS, in collaboration with other NIH Institutes and Centers and the Food and Drug Administration (FDA), is leading the Tissue Chip for Drug Screening program to develop human tissue chips that accurately model the structure and function of human organs — such as the lungs, liver and heart — to help predict drug safety in humans more rapidly and effectively. During the program’s inception, it has focused on developing physiologically relevant models for toxicity testing. The current focus of the program is on disease modelling and efficacy testing.

	Once developed and integrated, researchers can use these models to predict whether a candidate drug, vaccine or biologic agent is safe or toxic in humans in a faster and more effective way than current methods. The ultimate goal of the program and Consortium is to accelerate the translation of basic discoveries into the clinic. By creating an integrated human body-on-a-chip, researchers can test the varied potential effects of a substance such as a drug across the entire body before any testing in humans.
Category	Development Opportunity Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	This building block can be used in the early phases of drug development, from disease knowledge discovery to product discovery. It can also be used during early clinical trials to inform patient-relevant decision-making. Interested parties (academic/industry drug developers, patient advocacy groups) can make contact with funded investigators within the Tissue Chip Consortium and potentially form collaborations with them. The systems database is now called ByoSystics (https://mps.csb.pitt.edu/) and has consolidated the information gleaned from the projects within the Consortium that can be utilized by biomedical researchers.
Stakeholders	- Funded Tissue Chip Consortium investigators - NCATS program staff who manage the Tissue Chip program
Enablers/ Requirements	A collaboration with Tissue Chip Consortium investigators or the database; users may need iPSCs or donor samples of tissue, or other relevant sources of data, e.g. clinical data.
Output	A collaboration with Tissue Chip Consortium investigators to enable further research into the disease or body system, e.g. creation of an in vitro tool to test multiple drugs efficiently.
Best time to apply and time window	The tool has its best use in the very early phases of drug development, in particular in basic research or preclinical development stage.

Expert tips	Contact program staff to find out more (info@ncats.nih.gov). Different organs and tissues are at different stages of modeling on tissue chips, so there may not be available tissue chips yet, or they may be at very early stages of development. PROs: Efficient way to test efficacy and safety without involving animals or vulnerable patient populations, thereby reducing development cost, while accurately modeling the structure and function of human organs. CONS: Tissue Chips for drug development are still a relatively new tool, therefore knowledge on their use is somewhat limited. Their use for a specific purpose in drug development should be confirmed as acceptable with the relevant Competent Authority.
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