Rapidly advancing pipeline of investigational precision therapies

DRUG CANDIDATE (TARGET)	Discovery	Early stage clinical development	Late stage clinical development	Regulatory submission	Approved	Commercial rights
Avapritinib (KIT & PDGFRA)	PDGFRA mutant GIST[1]			NDA/MAA submitted		
	4L GIST[1]			NDA/MAA submitted		
	3L GIST[1]			NDA planned 2020		
	2L GIST[1]		Trial planned 2H 2019			
	Advanced SM			NDA planned Q1 2020		
	Indolent and smoldering SM					
Pralsetinib *formerly BLU-667*	2L RET-fusion NSCLC[1]			NDA planned Q1 2020		**
	1L RET-fusion NSCLC[1] – Trial planned 2H 2019					
	EGFR-m NSCLC (+osimertinib)[1] – Trial planned 2H 2019					
	2L RET-mutant MTC[1]			NDA planned 1H 2020		
	Other RET-altered solid tumors[1]					
Fisogatinib *formerly BLU-554*	Advanced HCC					
	Advanced HCC (+CS-1001) – Trial planned 2H 2019					
BLU-782 (ALK2)	FOP[2]					
4 wholly owned programs	Undisclosed targets					
Cancer immunotherapy	Up to 5 cancer immunotherapy programs; development stage undisclosed					Roche **

EGFR-m, EGFR mutant; FOP, fibrodysplasia ossificans progressiva; HCC, hepatocellular carcinoma. [1]Unresectable or metastatic disease. [2]Phase 1 trial in healthy volunteers ongoing; phase 2 trial in patients with FOP planned Q4 2019.

*CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains all rights in the rest of the world.

**Blueprint Medicines has U.S. commercial rights for up to two programs. Roche has worldwide commercialization rights for up to three programs and ex-U.S. commercialization rights for up to two programs.