Rapidly advancing pipeline

Genomically defined cancers

DISCOVERY | EARLY-STAGE DEVELOPMENT | LATE-STAGE DEVELOPMENT | REGULATORY SUBMISSION | APPROVED

Avapritinib: PDGFRA

- PDGFRA GIST[1,2,3] — MAA; U.S.

Pralsetinib: RET

- RET+ NSCLC[1,2,4,5] — MAA; U.S.
- EGFR+ NSCLC (+osimertinib)[1,2,4]
- RET+ MTC[1,2,4] — NDA
- RET+ thyroid cancer[1,2,4] — NDA
- Other RET-altered solid tumors[1,2,4]

Fisogatinib: FGFR4

- Advanced HCC[2]
- Advanced HCC (+CS1001)[2]

BLU-945: EGFR+ T790M/C797S triple mutant

- EGFR+ NSCLC[1]

Research program: EGFR+ C797S double mutant

- EGFR+ NSCLC[1]

Research programs: 2 undisclosed targets

Rare diseases

Avapritinib: KIT

- Advanced SM[2] — NDA
- Indolent SM[2]

BLU-263: KIT

- Indolent SM

Cancer immunotherapy

Research program: MAP4K1[6]

Research programs: 3 undisclosed targets[6]

ongoing or completed | planned

Updated as of September 4, 2020.

[1]Unresectable or metastatic disease.

[2]CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan.

[3]Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. The proposed indication for the MAA is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. In July 2020, received a positive opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use.

[4]In collaboration with Roche. Blueprint Medicines and Roche have co-exclusive rights to develop and commercialize pralsetinib in the U.S., and Roche has exclusive rights to develop and commercialize pralsetinib outside the U.S., excluding the CStone territory.

[5]Received accelerated approval in the U.S. for the treatment of adults with metastatic RET fusion-positive NSCLC. Continued approval may be contingent on a confirmatory trial. The proposed indication for the MAA is locally advanced or metastatic RET fusion-positive NSCLC previously treated with platinum-based chemotherapy.

[6]In collaboration with Roche. Blueprint Medicines has U.S. commercial rights for up to two programs. Roche has worldwide commercialization rights for up to two programs and ex-U.S. commercialization rights for up to two programs.

GIST = gastrointestinal stromal tumors. HCC = hepatocellular carcinoma. MAA = marketing authorization application.
MTC = medullary thyroid cancer. NDA = new drug application. NSCLC = non-small cell lung cancer. SM = systemic mastocytosis.

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