Biodexa Licenses Phase 1 Ready Drug Candidate from Otsuka for Rare Stomach Cancer

MTX240's mechanistic novelty may give it a long-awaited edge in treatments for gastrointestinal stromal tumors (GIST)

Biodexa Pharmaceuticals PLC (NASDAQ: BDRX), a clinical-stage biopharmaceutical company developing a pipeline of innovative products for the treatment of rare diseases with an increasing focus on products to treat or prevent gastrointestinal cancers, has added a new Phase 1 ready candidate to its portfolio, this time to target gastrointestinal stromal tumors, or GIST.

Several drugs are available to treat GIST, but all ultimately fail when the tumor develops resistance. When that occurs, median overall survival drops to less than a year. The value and hope of a better drug is at least partially evidenced by the eye-popping $1

billion GSK paid last year for a Phase 1 candidate, even though it shares the same mechanism of action as the currently approved drugs.

MTX240's unique molecular glue mechanism of action separates it from the current standard of care, notes Stephen Stamp, CEO of Biodexa. "It has the potential to benefit a broader spectrum of GIST patients, including those with TKI-resistant disease, and it strategically aligns with our emerging GI/oncology pipeline which includes our ongoing phase 3 development of eRapa in Familial Adenomatous Polyposis."

What is GIST?

GIST is a hard-to-treat tumor that grows mainly in the stomach wall and is caused by mutations in the so-called Interstitial Cells of Cajal (ICCs) which facilitate gut motility. Mutations in the key tyrosine kinase proteins KIT or PDGFR perturb intra-cellular signaling pathways in ICCs, causing rapid cell proliferation and tumorgenesis. Patients typically undergo resection but, once the GIST becomes unmanageable or the tumors are unresectable, targeted treatments called tyrosine kinase inhibitors (TKIs) are the current standard of care.

Current standard of care

Currently, there are four approved TKI treatments for GIST, generally prescribed in the following order as GIST mutations occur and patients acquire resistance to each of them – (1) imatinib (Gleevec) from **Novartis AG** (NYSE: NVS); (2) sunitinib (Sutent) from **Pfizer Inc.** (NYSE: PFE); (3) regorafenib (Stivarga) from **Bayer AG**. (DE: BAYN); and (4) ripretinib (Qinlock) from **Deciphera Pharmaceuticals Inc** (NASDAQ: DCPH).

Once patients have cycled through the available TKIs, having acquired resistance to all of them, median time to tumor progression is 1.2 months and median overall survival is 9.8 months.

As a result of these shortcomings, drug developers, including Biodexa, are jockeying for position in what is currently a $1.3 billion market[1], with projected growth of around 6.6% annually through 2032. It makes sense that drug developers are eyeing this market. In the U.S. alone, between 4,000 and 6,000 people are diagnosed with GIST each year [2]. Globally, the number of new GIST patient annually is estimated at between 80,000 to 120,000.

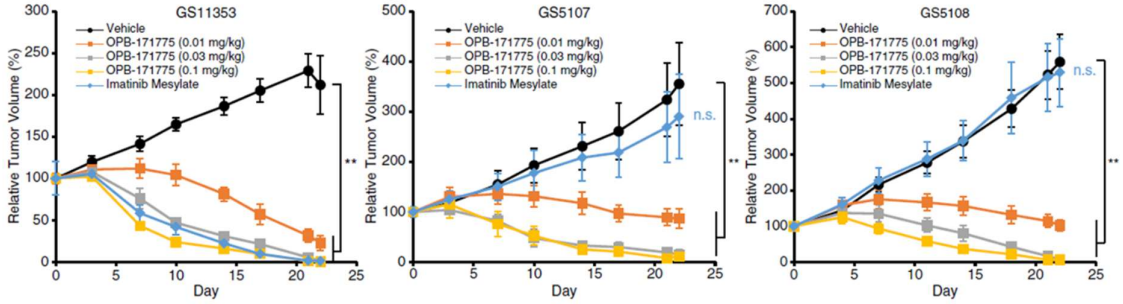
Biodexa's novel drug candidate – MTX240

Enter MTX240, Biodexa's latest developmental treatment, which it recently licensed from **Otsuka Pharmaceutical Co., Ltd.** MTX240 is a novel molecular glue that works by forcing PDE3A and SLFN12, two proteins expressed in GIST cells to bind together, in turn triggering the tumor cells to self-destruct in a process called apoptosis. Importantly, healthy ICCs do not significantly express either of these proteins and are not therefore affected by MTX240.

Unlike the TKIs that frequently fail once the tumor cells mutate, and render the drug ineffective, MTX240's unique mechanism of action is designed to work whether or not the GIST has acquired secondary mutations.

Early success and promise

In preclinical PDX models, which use human tumor tissues to mirror real-world genetic complexity, MTX240 demonstrated dose-dependent efficacy in shrinking GIST in both non-resistant (left hand chart) and TKI-resistant tumors (middle and right hand chart) as illustrated below [3]:



Note: MTX240 was coded OPB-171775 by Otsuka in the charts.

If the preclinical results are replicated in clinical trials and MTX240 proves to be lethal to GIST without inducing resistance, it has the potential for co-administration with TKIs and ultimately, to replace the entire class.

MTX240 is eligible for FDA and EU Orphan Drug designation, which means the company is eligible for seven and 10 years of market exclusivity upon approval in the US and EU, respectively. In addition, MTX240 has issued Composition of Matter patents extending through 2037.

Next steps

Next step is to manufacture clinical trial supplies of MTX240 and then initiate a Phase 1b/2a study by year-end. The study is expected to be in two parts: a standard dose escalation part to determine a maximum tolerated dose, followed by an extension part. The extension part is likely to enrol patients with TKI-resistant GIST. By focusing on this high-need population, Biodexa is aiming to rapidly validate MTX240's potential as a way to treat patients who don't respond to the current standards of care.

Deal validation

Biodexa in-licensed exclusive worldwide rights to MTX240 with the exception of Japan rights, which were retained by Otsuka for an undisclosed upfront fee. The deal includes one modest development milestone, low double digit approval milestones and mid single-digit tiered royalties.

Biodexa's entrance into the GIST market with MTX240 puts it in a space that's garnering interest from Big Pharma as well as Wall Street. Take **GSK Plc.** (NYSE: GSK) as one example. In January 2025 GSK paid $1 billion cash upfront to buy IDRx, Inc., a single product company developing IDRX-42, another TKI for GIST that was in Phase 1 at the time [4]. Meanwhile, **Cogent Biosciences Inc.** (NASDAQ: COGT), which is developing bezuclastinib (another TKI) for co-administration with sunitinib, saw its market cap surge approximately $2Bn following the release of Phase 3 trial data [5], which demonstrated bezuclastinib plus sunitinib compared with sunitinib alone extended median progression free survival from 9.2 months to 16.5 months in imatinib-resistant patients, underscoring the importance of this sector [6].

With Biodexa showing promising initial results with MTX240, this is a treatment investors may want to pay attention to. To learn more about Biodexa and MTX240, click here.

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Contact:
Stephen Stamp, CEO
ir@biodexapharma.com

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