



NEWS RELEASE

bridgebio pharma, inc. reports third quarter 2022 financial results and business update

2022-11-03

- Reported positive preclinical data for its next-generation KRAS^{G12C} GTP/GDP dual inhibitor development candidate, BBO-8520, and for its novel PI3K α :RAS breaker mechanism in late lead optimization
- Reported positive updated 12-month Phase 2 data for BBP-418 in Limb-Girdle Muscular Dystrophy Type 2i
- Enrolled Cohort 5 of Phase 2 trial of infigratinib in achondroplasia, with no serious adverse events (SAEs), and no adverse events that required dose modifications reported to date
- Reported dosing of first lung cancer patient in Phase 1/2 trial of SHP2 inhibitor BBP-398 in combination with Amgen's Lumakras (sotorasib); also reported dosing of first patient in Phase 1 trial of BBP-671 in Propionic Acidemia (PA) and Methylmalonic Acidemia (MMA)
- Reported updated positive data from its Phase 1/2 trial of BBP-812, an AAV9 gene therapy candidate for the treatment of Canavan disease
- Received approval of NULIBRY (fosdenopterin) as a treatment for molybdenum cofactor deficiency (MoCD) Type A in Israel and the European Union (EU)
- Reported operating expense for Q3 2022 at \$129.5 million, a reduction of 26.2% from \$175.4 million in Q1 2022 when restructuring efforts began, and a reduction of 15.9% from \$153.9 million in Q2 2022.

- Ended quarter with \$558.3 million in cash, cash equivalents and marketable securities, providing financial runway into 2024

PALO ALTO, Calif., Nov. 03, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio or the Company), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today reported its financial results for the third quarter ended September 30, 2022 and provided an update on the Company's operations.

"We remain committed to focused execution across our pipeline, and have continued to deliver exciting data for patients, including in our LGMD2i and KRAS programs, while at the same time materially reducing our operating expenses and putting the Company on safe footing," said Neil Kumar, Ph.D., founder and CEO of BridgeBio.

"The compounds that we have developed in our RAS franchise after years of intensive work now allow us to clinically interrogate the promise of direct inhibition of active KRAS, as well as the potential of breaking PI3K α :RAS binding," added Frank McCormick, Ph.D., Chairman of Oncology and cofounder of BridgeBio. "RAS and PIK3CA are the two most common oncogenes in human tumors, and we hope that our programs are able to serve the vast unmet need of the patients that they impact."

BridgeBio's Key Programs

RAS cancer portfolio:

- BridgeBio has selected a next-generation KRAS^{G12C} dual inhibitor development candidate, BBO-8520, and plans to be in the clinic in 2023.
- The Company shared promising preclinical data on BBO-8520, as well as its novel PI3K α :RAS breaker program in late lead optimization, at the Fourth RAS Initiative Symposium.
- BBO-8520 has shown significantly greater potency in KRAS models than first-generation KRAS^{G12C} GDP-only inhibitors as measured by its ability to bind and covalently modify KRAS^{G12C}, block KRAS^{G12C} binding to effector proteins such as RAF, and inhibit downstream signaling.
- BBO-8520 was shown to retain potency in the context of receptor tyrosine kinase drive, which renders KRAS^{G12C} GDP-only inhibitors inactive and is thought to be a major mechanism of non-response and resistance to first-generation agents.
- BBO-8520 showed strong activity in KRAS^{G12C} in vivo models including deep regressions and differentiated efficacy compared to a first-generation KRAS^{G12C} GDP-only inhibitor.
- BridgeBio scientists highlighted rationale and design of compounds targeting PI3K α :RAS binding, which is a

novel and potentially broad MoA to target PI3K α mutant tumors, RAS mutant tumors and potentially other tumors driven by RTK activation of RAS signaling.

- Targeting PI3K α activity in tumors through its interaction with RAS may spare glucose metabolism, potentially allowing for potent target coverage without displaying the dose-limiting hyperglycemia common to PI3K α kinase inhibitors.
- RAS is the most common oncogenic driver with approximately 30% of all human cancers being driven by RAS mutations, including large proportions of lung, colorectal and pancreatic tumors. PIK3CA is the second most common oncogene in human tumors, being present in more than 30% of breast and endometrial carcinomas.

Low-dose infigratinib – FGFR1-3 inhibitor for achondroplasia and hypochondroplasia:

- Earlier this year BridgeBio shared **positive interim results from available data in Cohort 4** from a Phase 2 trial of low-dose infigratinib in patients with achondroplasia, which demonstrated an increase in annualized height velocity of 1.52 cm/year in children 5 years of age and older.
- Given infigratinib's profile to date, and after discussions with regulators, BridgeBio started dosing children in Cohort 5; that cohort is now enrolled with no serious adverse events (SAEs), and no adverse events that required dose modifications reported to date.
- The Company expects to report an update on Cohort 5 AHV in the first half of 2023, followed by the initiation of a pivotal Phase 3 trial.
- With more than 55,000 cases estimated in the United States (US) and Europe, achondroplasia is the most common form of genetic short stature and one of the most common genetic conditions. BridgeBio also expects to evaluate development of infigratinib in other FGFR-driven skeletal dysplasias, which affect more than 50,000 people in the US and Europe.

Encaleret – Calcium-sensing receptor (CaSR) inhibitor for autosomal dominant hypocalcemia type 1 (ADH1):

- BridgeBio intends to initiate a Phase 3 pivotal study of encaleret in patients with ADH1 by the end of 2022 and expects to release topline data by year-end 2023.
- Positive data from the Company's Phase 2b study of encaleret in ADH1 were shared earlier this year in an **oral presentation** at the Endocrine Society's 2022 ENDO Conference.
- The Phase 2b study demonstrated that treatment with encaleret resulted in rapid and sustained restoration of normal mineral homeostasis by day 5 of therapy which sustained at 24 weeks, and encaleret was well-tolerated without any reported SAEs.
- If approved, encaleret could be the first therapy indicated for the treatment of ADH1, a condition caused by gain of function variants of the CASR gene estimated to be carried by 12,000 individuals in the United States alone.

BBP-418 – Glycosylation substrate for limb-girdle muscular dystrophy type 2i (LGMD2i):

- The Company shared updated positive Phase 2 data in a **presentation** at the World Muscle Society (WMS) 27th International Hybrid Annual Congress
- BridgeBio engaged with regulatory health bodies to align on a Phase 3 trial design and intends to initiate a Phase 3 clinical trial in the first half of 2023
- Phase 2 results indicate the potential for BBP-418 to increase glycosylation of alpha-dystroglycan (α DG), which is directly linked to the underlying disease mechanism, and to drive consistent improvements of muscle function in patients as measured by the reduction of creatine kinase, a key marker of muscle breakdown
- 12-month data show improvements from baseline on 10-meter walk test and North Star Assessment for Dysferlinopathy, which BridgeBio believes suggests a potential impact on clinical function and on the rate of disease progression
- If proven to be successful, BBP-418 could be the first approved therapy for patients with LGMD2i

Acoramidis (AG10) – Transthyretin (TTR) stabilizer for transthyretin amyloid cardiomyopathy (ATTR-CM):

- The Phase 3 ATTRIBUTE-CM trial is progressing with topline data from the Month 30 primary endpoint, a hierarchical composite including all-cause mortality and cardiovascular hospitalizations, expected in mid-2023
- The Company **presented** updated results of the Phase 2 open-label extension of acoramidis in ATTR-CM, demonstrating near-complete TTR stabilization and stable or improving serum NT-proBNP levels at month 30

BBP-631 – AAV5 gene therapy candidate for congenital adrenal hyperplasia (CAH):

- The Phase 1/2 study is ongoing with an update anticipated late in 2022 or early in 2023.
- With more than 75,000 patients estimated in the US and EU, CAH is one of the most prevalent genetic diseases potentially addressable with adeno-associated virus (AAV) gene therapy
- The disease is caused by deleterious mutations in the gene encoding an enzyme called 21-hydroxylase, leading to a lack of endogenous cortisol production
- BBP-631 is designed to provide a functional copy of the 21-hydroxylase-encoding gene (CYP21A2) and potentially address many aspects of the disease course
- If successful, BridgeBio's investigational gene therapy would be the first therapy for CAH to restore the body's hormone and steroid balance by enabling people with CAH to make their own cortisol and aldosterone

Recent Corporate Updates

- Updated positive data for investigational AAV9 gene therapy in Canavan disease: Promising pharmacodynamic data from the first three participants dosed in the Phase 1/2 clinical trial of BBP-812 for the treatment of Canavan disease. Results showed unprecedented decreases in N-acetylaspartate (NAA) in the

brain and urine, suggesting the therapy is producing functional ASPA enzyme. Affecting approximately 1,000 children in the United States and European Union, Canavan disease is an ultra-rare, disabling and fatal disease with no approved therapy.

- Approvals in Israel and EU for NULIBRY (fosdenopterin): The European Commission (EC) granted marketing authorization for NULIBRY Powder for Solution for Injection as the first therapy for the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A in Europe, and the State of Israel Ministry of Health approved NULIBRY for Injection as a treatment for MoCD Type A patients in Israel. These decisions are based on the efficacy and safety data collected compared to data from a natural history study. NULIBRY is the first and only approved therapy in either region to treat patients with MoCD Type A, an ultra-rare, life-threatening genetic disorder that often progresses rapidly in infants with a median overall survival age of about four years. NULIBRY was BridgeBio's first FDA-approved and EC-approved therapeutic. Medison Pharma acquired commercialization rights to NULIBRY in Israel in December 2019. Sentyln Therapeutics, Inc. acquired global rights to NULIBRY in March 2022.
- First patient dosed in Phase 1/2 combination trial of BridgeBio's SHP2 inhibitor BBP-398 with Amgen's LUMAKRAS: First lung cancer patient was dosed in a Phase 1/2 trial of BridgeBio's SHP2 inhibitor BBP-398 in combination with Amgen's LUMAKRAS (sotorasib) in advanced solid tumors with the KRAS^{G12C} mutation; additionally, FDA Fast Track Designation was obtained for BBP-398 in combination with LUMAKRAS for adult patients with previously treated, KRAS^{G12C} mutated, metastatic non-small-cell lung cancer (NSCLC). BridgeBio has a non-exclusive clinical collaboration with Amgen to evaluate the combination of BBP-398 with LUMAKRAS in patients with advanced solid tumors with the KRAS^{G12C} mutation. BridgeBio is party to an exclusive license agreement with Bristol Myers Squibb (BMS) to develop and commercialize BBP-398 in oncology worldwide, except for in mainland China and other Asian markets, which are part of BridgeBio's strategic collaboration with LianBio. BridgeBio and BMS are also investigating the combination of BBP-398 with OPDIVO® (nivolumab) in patients with advanced solid tumors with KRAS mutations.
- First patient dosed in Phase 1 trial of BBP-671 for propionic acidemia (PA) and methylmalonic acidemia (MMA): An initial data readout of patients with PA and MMA is expected in mid-2023. BridgeBio is also in active discussions with regulators and expects to launch a pivotal Phase 2/3 study of BBP-671 in pantothenate kinase-associated neurodegeneration (PKAN) in 2024. If successful, BBP-671 has potential to be a best-in-class therapy for PA, MMA, and PKAN patients, as well as the first approved oral therapy for the treatment of systemic complications caused by CoA deficiencies. PA, MMA, and PKAN affect an estimated 7,000 patients in the United States and European Union collectively.

Third Quarter 2022 Financial Results:

Cash, Cash Equivalents and Marketable Securities

Cash, cash equivalents and marketable securities, excluding restricted cash, totaled \$558.3 million as of September 30, 2022, compared to \$787.5 million as of December 31, 2021. The net decrease of \$229.2 million in cash, cash equivalents and marketable securities, excluding restricted cash is primarily attributable to net cash used in operating activities of \$326.3 million. The net cash used in operating activities for the nine months ended September 30, 2022 was partially offset by a \$90.0 million in upfront payment received under the License, Development and Commercialization Agreement between the Company, its affiliate, Navire Pharma, Inc., and Bristol Myers Squibb (the "Navire-BMS License Agreement"). During the nine months ended September 30, 2022, the Company also received upfront payments of \$110.0 million from the sale of its priority review voucher and \$10.0 million upon closing of an asset purchase agreement between its affiliate, Origin Biosciences, Inc., and Sentyln Therapeutics, Inc. The Company made a \$20.5 million mandatory prepayment of a portion of its term loan obligations under its Amended Loan and Security Agreement in connection with the upfront payment received from BMS.

Cash, cash equivalents and marketable securities, excluding restricted cash, decreased by \$130.3 million when compared to the balance as of June 30, 2022 of \$688.6 million. Net cash used in operating activities, was \$135.2 million for the three months ended September 30, 2022. Net cash used in operating activities was \$191.1 million for the six months ended June 30, 2022.

Operating Costs and Expenses

Operating costs and expenses for the three and nine months ended September 30, 2022 were \$129.5 million and \$458.7 million, respectively, as compared to \$151.8 million and \$467.8 million for the same periods in the prior year. The overall decrease in operating costs and expenses for the three and nine months ended September 30, 2022 compared to the comparative periods was mainly due to overall decreases in selling, general and administrative expenses and research, development and other (R&D) expenses resulting from the Company's reprioritization of its R&D programs and streamlining of costs. The effects of the Company's restructuring initiative that was started in the first quarter of 2022 are now being realized due to reductions of its operating costs and expenses. Restructuring, impairment and related charges for the three and nine months ended September 30, 2022 of \$5.0 million and \$36.1 million, respectively, were primarily comprised of winding down costs, exit and other related costs, impairments and write-offs of long-lived assets, and severance and employee-related costs. The Company continues to evaluate restructuring alternatives to drive operational changes in business processes, efficiencies, and cost savings.

"We continue to take action to protect the Company, shore up our balance sheet, and preserve capital to read out our upcoming key catalysts," said Brian Stephenson, Ph.D., CFA, Chief Financial Officer of BridgeBio. "We expect that cash burn will continue to decline in the fourth quarter as a result of ongoing potential business development and

restructuring activities. Cash on hand provides us with runway into 2024, and we will continue to look for ways to extend runway via potential royalty monetizations, partnerships, and burn reduction.”

The Company’s research and development and other expenses have not been significantly impacted by the global COVID-19 pandemic for the periods presented. While BridgeBio experienced some delays in certain of its clinical enrollment and trial commencement activities, it continues to adapt with alternative site, telehealth and home visits, and at-home drug delivery, as well as mitigation strategies with its contract manufacturing organizations. The longer-term impact, if any, of COVID-19 on BridgeBio’s operating costs and expenses is currently unknown.

BRIDGEBIO PHARMA, INC.
Condensed Consolidated Statements of Operations
(in thousands, except shares and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
	(Unaudited)		(Unaudited)	
Revenue	\$ 338	\$ 2,344	\$ 75,778	\$ 56,830
Operating costs and expenses:				
Research, development and others	93,250	105,759	311,347	330,387
Selling, general and administrative	31,188	46,084	111,327	137,461
Restructuring, impairment and related charges	5,016	—	36,074	—
Total operating costs and expenses	<u>129,454</u>	<u>151,843</u>	<u>458,748</u>	<u>467,848</u>
Loss from operations	(129,116)	(149,499)	(382,970)	(411,018)
Other income (expense), net:				
Interest income	2,417	234	3,450	951
Interest expense	(19,825)	(11,067)	(60,448)	(31,644)
Gain from sale of priority review voucher, net	—	—	107,946	—
Other income (expense), net	6,331	(684)	(12,060)	7,539
Total other income (expense), net	<u>(11,077)</u>	<u>(11,517)</u>	<u>38,888</u>	<u>(23,154)</u>
Net loss	(140,193)	(161,016)	(344,082)	(434,172)
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	2,854	5,081	490	18,810
Net loss attributable to common stockholders of BridgeBio	<u>\$ (137,339)</u>	<u>\$ (155,935)</u>	<u>\$ (343,592)</u>	<u>\$ (415,362)</u>
Net loss per share, basic and diluted	<u>\$ (0.93)</u>	<u>\$ (1.06)</u>	<u>\$ (2.34)</u>	<u>\$ (2.88)</u>
Weighted-average shares used in computing net loss per share, basic and diluted	<u>147,937,817</u>	<u>146,662,756</u>	<u>146,842,453</u>	<u>144,044,360</u>
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
	(Unaudited)		(Unaudited)	
Research, development and others	\$ 6,137	\$ 4,808	\$ 29,046	\$ 46,541
Selling, general and administrative	12,521	11,322	41,026	36,520
Restructuring, impairment and related charges	—	—	1,172	—
Total stock-based compensation	<u>\$ 18,658</u>	<u>\$ 16,130</u>	<u>\$ 71,244</u>	<u>\$ 83,061</u>

BRIDGEBIO PHARMA, INC.
Condensed Consolidated Balance Sheets
(In thousands)

	September 30, 2022	December 31, 2021
Assets	(Unaudited)	(1)
Cash and cash equivalents and marketable securities	\$ 558,315	\$ 787,515
Investment in equity securities	33,662	49,148
Receivable from licensing and collaboration agreements	24,581	19,749
Prepaid expenses and other current assets	25,661	32,446
Property and equipment, net	15,603	30,066
Operating lease right-of-use assets	11,738	15,907
Intangible assets, net	29,310	44,934
Other assets	29,870	33,027
Total assets	<u>\$ 728,740</u>	<u>\$ 1,012,792</u>
Liabilities, Redeemable Convertible Noncontrolling Interests and Stockholders' Deficit		
Accounts payable	\$ 10,158	\$ 11,884
Accrued and other liabilities	104,992	118,247
Operating lease liabilities	17,044	22,366
2029 Notes	734,516	733,119
2027 Notes	541,205	539,934
Term loans	422,972	430,752
Other long-term liabilities	28,226	22,069
Redeemable convertible noncontrolling interests	(2,388)	1,423
Total BridgeBio stockholders' deficit	(1,138,417)	(870,414)
Noncontrolling interests	10,432	3,412
Total liabilities, redeemable convertible noncontrolling interests and stockholders' deficit	<u>\$ 728,740</u>	<u>\$ 1,012,792</u>

(1)The condensed consolidated financial statements as of and for the year ended December 31, 2021 are derived from the audited consolidated financial statements as of that date.

BRIDGEBIO PHARMA, INC.
Condensed Consolidated Statements of Cash Flows
(In thousands)

	Nine Months Ended September 30,	
	2022	2021
	(Unaudited)	
Operating activities:		
Net loss	\$ (344,082)	\$ (434,172)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	69,770	79,731
Depreciation and amortization	5,111	4,317
Net loss from investment in equity securities	12,969	1,510
Gain from sale of priority review voucher, excluding transaction costs	(110,000)	—
Gain from recognition of receivable from licensing and collaboration agreement	(12,500)	—
Fair value of shares issued under a license agreement	4,567	—
Accretion of debt	6,469	4,043
Fair value adjustment of warrants	1,446	459
Loss on sale of certain assets	6,261	—
Impairment of long-lived assets	12,720	3,300
LEO call option income	—	(5,550)
Other noncash adjustments	4,687	7,322
Changes in operating assets and liabilities:		
Receivable from licensing and collaboration agreements	(832)	(7,710)
Receivable from a related party	—	(462)
Prepaid expenses and other current assets	4,072	(3,743)
Other assets	10,095	(8,930)
Accounts payable	(1,725)	1,360
Accrued compensation and benefits	(9,122)	(4,443)
Accrued research and development liabilities	452	4,686
Accrued professional services	(2,556)	346
Operating lease liabilities	(4,819)	(4,474)
Deferred revenue	16,969	—
Other accrued and other long-term liabilities	3,797	(1,629)
Net cash used in operating activities	<u>(326,251)</u>	<u>(364,039)</u>
Investing activities:		
Purchases of marketable securities	(134,635)	(575,478)
Maturities of marketable securities	452,819	305,200
Sales of marketable securities	—	98,925
Purchases of investment in equity securities	(26,312)	(23,960)
Sales of investment in equity securities	28,830	4,743
Increase in cash and cash equivalents from consolidation of PellePharm	—	13,654
Acquisition and payment of an intangible asset	(1,500)	(35,000)
Proceeds from sale of priority review voucher	110,000	—
Proceeds from sale of certain assets	10,000	—
Purchases of property and equipment	(4,020)	(10,710)
Net cash provided by (used in) investing activities	<u>435,182</u>	<u>(222,626)</u>
Financing activities:		
Proceeds from issuance of 2029 Notes	—	747,500
Issuance costs and discounts associated with issuance of 2029 Notes	—	(16,064)
Issuance costs associated with term loan	(1,120)	—
Purchase of capped calls	—	(61,295)
Repurchases of common stock	—	(198,458)
Transactions with noncontrolling interests	—	3,500
Repurchase of Eidos noncontrolling interest, including direct transaction costs	—	(85,090)
Proceeds from term loan	—	25,000
Repayment of term loan	(20,486)	(18,108)
Proceeds from BridgeBio common stock issuances under ESPP	2,558	3,821
Repurchase of shares to satisfy tax withholding	(1,072)	(4,035)
Proceeds from stock option exercises, net of repurchases	609	14,294
Net cash provided by (used in) financing activities	<u>(19,511)</u>	<u>411,065</u>
Net increase in cash, cash equivalents and restricted cash	89,420	(175,600)
Cash, cash equivalents and restricted cash at beginning of period	<u>396,365</u>	<u>358,679</u>
Cash, cash equivalents and restricted cash at end of period	<u>\$ 485,785</u>	<u>\$ 183,079</u>

	Nine Months Ended September 30,	
	2022	2021
Supplemental Disclosures of Cash Flow Information:		
Cash paid for interest	\$ 47,575	\$ 28,239
Supplemental Disclosures of Noncash Investing and Financing Information:		
Payment-in-kind interest added to principal of term loan	\$ 8,503	\$ —
Net noncash portion of repurchase of Eidos noncontrolling interests	\$ —	\$ 38,167
Direct transaction costs in the repurchase of Eidos recorded in "Additional paid-in capital" previously classified in "Prepaid expenses and other current assets"	\$ —	\$ 8,749
Noncash contribution by a noncontrolling interest	\$ —	\$ 21,600
Recognized intangible asset recorded in "Accrued research and development liabilities"	\$ 11,000	\$ 12,500
Leasehold improvements paid by landlord	\$ —	\$ 2,449
Repurchase of common stock recorded in Accounts payable	\$ —	\$ 1,542
Transfers from noncontrolling interests	\$ 1,153	\$ (221)
Reconciliation of Cash, Cash Equivalents and Restricted Cash:		
Cash and cash equivalents	\$ 483,235	\$ 180,347

Restricted cash — Included in "Prepaid expenses and other current assets"
 Restricted cash — Included in "Other assets"
 Total cash, cash equivalents and restricted cash at end of period shown in the condensed consolidated statements of cash flows

	140		1 / 6
	2,410		2,556
\$	485,785	\$	183,079

About BridgeBio Pharma, Inc.

BridgeBio Pharma, Inc. (BridgeBio) is a commercial-stage biopharmaceutical company founded to discover, create, test and deliver transformative medicines to treat patients who suffer from genetic diseases and cancers with clear genetic drivers. BridgeBio's pipeline of development programs ranges from early science to advanced clinical trials. BridgeBio was founded in 2015 and its team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit bridgebio.com and follow us on [LinkedIn](#) and [Twitter](#).

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act), which are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act. These forward-looking statements, including statements relating to the clinical and therapeutic potential of our programs and product candidates, including the timing and success of our RAS program, including preclinical data for our next-generation KRASG12C GTP/GDP dual inhibitor development candidate, BBO-8520 and plans to be in the clinic in mid-2023, updated data from our Phase 2 study of BBP-418 for patients LGMD2i, the timing and success of regulatory discussions regarding potential paths to approval for BBP-418, the ability of BBP-418 to be the first approved therapy for patients with LGMD2i, the timing and success of a Phase 3 trial of BBP-418 in patients with LGMD2i intended to be initiated in the first half of 2023, the availability and success of data from our ongoing Phase 1/2 trial of SHP2 inhibitor BBP-398 in combination with Amgen's Lumakras (sotorasib), the availability and success of additional data from our ongoing Phase 1/2 trial of BBP-812 for the treatment of Canavan disease, the availability and success of additional data from our ongoing Phase 1 study of BBP-671 for PKAN and organic acidemias, the approval of NULIBRY (fosdenopterin) for treatment of MoCD Type A in Israel and the EU, the availability and success of initial data from our ongoing Phase 2 study of low-dose infigratinib for achondroplasia, including plans to deliver an update on Cohort 5 in the first half of 2023, followed by the initiation of a pivotal Phase 3 trial, the evaluation of the development of infigratinib in other FGFR-driven skeletal dysplasias, the availability and success of additional

data from our ongoing Phase 2b study of encalaret for ADH1, the timing and success of additional trials of encalaret for ADH1, including the timing and announced design of a Phase 3 pivotal study of encalaret for ADH1, the timing and success of our planned Phase 3 pivotal study of encalaret in patients with ADH1, the availability and success of topline results from the Part B Month 30 endpoint of our Phase 3 ATTRIBUTE-CM trial of acoramidis, expected in mid-2023, the availability and success of data from our ongoing Phase 1/2 study of BBP-631 for CAH, with an update anticipated in late 2023 or early 2023, the timing, availability and success of an initial data readout from our Phase 1 trial of BBP-671 in patients with PA and MMA expected in mid-2023, the timing and success of discussions with regulators and the expected launch of a pivotal Phase 2/3 study of BBP-671 in PKAN in 2024, the potential of BBP-671 to be a best-in-class therapy for PA, MMA, and PKAN patients, as well as the first approved oral therapy for the treatment of systemic complications caused by CoA deficiencies, if successful, the success of our license agreement with Bristol Myers Squibb to develop and commercialize BBP-398, including our eligibility for development, regulatory and sales milestone payments and tiered royalties, the success of our asset purchase agreement with Sentyln Therapeutics, including our ability to achieve future milestone and royalty payments from Sentyln Therapeutics and the timing of these events, the timing and success of partnering and out-licensing discussions for certain programs in our pipeline, the timing and availability of delayed debt draws under our senior secured credit facility, the success of our reduction in operating expenses and our expectations for our operating expenses and cash burn for the second quarter, the success of our restructuring initiative and its savings being realized, as well as our anticipated cash runway, reflect our current views about our plans, intentions, expectations and strategies, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations and strategies as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a number of risks, uncertainties and assumptions, including, but not limited to, initial and ongoing data from our preclinical studies and clinical trials not being indicative of final data, the potential size of the target patient populations our product candidates are designed to treat not being as large as anticipated, the design and success of ongoing and planned clinical trials, future regulatory filings, approvals and/or sales, despite having ongoing and future interactions with the FDA or other regulatory agencies to discuss potential paths to registration for our product candidates, the FDA or such other regulatory agencies not agreeing with our regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted, the continuing success of our collaborations, the Company's ability to unlock additional funding under our credit facility, potential volatility in our share price, potential adverse impacts due to the global COVID-19 pandemic such as delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, as well as those risks set forth in the Risk Factors section of our Annual Report on Form 10-K for the year ended December 31, 2021 and our other filings with the U.S. Securities and Exchange Commission. Moreover, we operate in a very competitive and rapidly changing environment in which new risks emerge from time to time. These

forward-looking statements are based upon the current expectations and beliefs of our management as of the date of this press release, and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. Except as required by applicable law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: BridgeBio Pharma, Inc.