



Ad hoc announcement pursuant to Art. 53 LR

Acer Therapeutics and Relief Therapeutics Announce U.S. FDA Approval of OLPRUVA™ for Patients with Urea Cycle Disorders

New FDA-approved formulation for patients living with urea cycle disorders

\$42.5 million of non-dilutive debt funding available to Acer that, if drawn, would extend cash runway into H2 2023

NEWTON, MASS. and GENEVA – Dec. 27, 2022 – [Acer Therapeutics Inc.](#) (Nasdaq: [ACER](#)) (“Acer”) and its collaboration partner, [RELIEF THERAPEUTICS Holding SA](#) (SIX: [RLF](#), OTCQB: [RLTFE](#), [RLFTY](#)) (“Relief”), today announced that the U.S. Food and Drug Administration (FDA) has approved OLPRUVA™ (sodium phenylbutyrate) for oral suspension in the U.S. for the treatment of certain patients living with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS).

“The FDA’s approval of OLPRUVA™, an innovative formulation of sodium phenylbutyrate packaged for the first time in single-dose envelopes, marks the culmination of our ongoing dedication to develop new and differentiated treatment options for those affected by rare diseases,” said Chris Schelling, chief executive officer and founder of Acer. “Patients who are living with UCDs now have an alternative treatment option with OLPRUVA™, to address some of the challenges they may have with existing therapy. We are pleased to be able to provide a new, approved treatment choice for those living with this challenging disease.”

Mr. Schelling continued, “This approval represents the first FDA-approved product for Acer, validating our ability to identify and develop treatments where science can be applied in novel ways and make them available to patients as quickly and efficiently as possible. In addition, this approval unlocks our Marathon debt funding option and provides us with resources to advance our pipeline of investigational product candidates.”

“The daily challenges of living with a UCD can be overwhelming and emotionally draining for patients and their families,” said Tresa Warner, president of the [National Urea Cycle Disorders Foundation](#). “We welcome new treatment options that can help patients, caregivers and their healthcare teams manage UCDs.”

OLPRUVA’s™ approval triggers the availability of a \$42.5 million term loan to Acer under the previously announced [March 2022](#) loan agreement the Company entered into with affiliates of Marathon Asset Management L.P. If Acer requests and receives the loan proceeds, management believes it will have sufficient resources to fund current operations into H2 2023.

OLPRUVA™ has been approved as an oral suspension by the FDA for the treatment of patients with UCDs. UCDs are a group of rare, genetic disorders that can cause harmful ammonia to build up in the blood, potentially resulting in brain damage and neurocognitive impairments, if ammonia levels are not controlled.¹ Any increase in ammonia over time is serious. Therefore, it is important to adhere to any dietary protein restrictions and have alternative medication options to help control ammonia levels.

“This FDA approval is a significant milestone for patients with UCDs in the U.S., offering an additional choice to manage their condition,” added Jack Weinstein, chief executive officer of Relief. “We look forward to building on OLPRUVA™’s approval in the U.S. and expanding its availability into other territories outside of the U.S.”

OLPRUVA™ received FDA approval under section 505(b)(2) of the Federal Food, Drug and Cosmetic Act (FDCA), a regulatory pathway that allows applicants to rely, at least in part, on third party data for approval. In its New Drug Application (NDA), Acer cited preclinical and clinical safety and efficacy data from the reference listed drug (RLD), BUPHENYL® powder, which is approved as adjunctive therapy in the chronic management of patients with UCDs involving deficiencies of CPS, OTC or AS. In its NDA, Acer also provided additional data including studies that evaluated the bioavailability and bioequivalence of OLPRUVA™ compared to BUPHENYL® powder. The data from these studies, [presented at the Society for Inherited Metabolic Disorders \(SIMD\) Annual Meeting in April 2022 and the Genetic Metabolic Dieticians International \(GMDI\) Conference in May 2022](#), showed that OLPRUVA™ was bioequivalent to BUPHENYL® powder.

Commitment to Patient Access

Acer intends to offer Navigator by Acer Therapeutics, a suite of integrated patient support services designed to facilitate access to therapy. Navigator by Acer Therapeutics is designed to assist UCD patients with support, access, education, and adherence.

Financial Outlook

Acer is not currently providing specific revenue or operating expense guidance for OLPRUVA™. Based on current forecasted operating expenses and revenue, and assuming receipt of the \$42.5 million term loan funds from its March 2022 term loan arrangement with Marathon (less the amount to repay the bridge loan and fees), and Acer’s existing cash and equivalents, Acer believes its cash resources will be sufficient to fund its operations into H2 2023. Further information with respect to Acer’s March 2022 term loan arrangement, as well as a bridge loan facility (as amended in August 2022) and a convertible note financing which also funded in March 2022 can be found in [the SEC Filings section of Acer’s website](#).

About OLPRUVA™

OLPRUVA™ is a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children weighing 44 pounds (20 kg) or greater and with a body surface area (BSA) of 1.2 m² or greater, with urea cycle disorders (UCDs), involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC) or argininosuccinic acid synthetase (AS). OLPRUVA™ is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment.

Important Safety Information

Certain medicines may increase the level of ammonia in your blood or cause serious side effects when taking during treatment with OLPRUVA™. Tell your doctor about all the medicines you or your child takes specially if you or your child takes corticosteroids, valproic acid, haloperidol, and/or probenecid.

OLPRUVA™ can cause serious side effects, including: 1) nervous system problems (neurotoxicity). Symptoms include sleepiness, tiredness, lightheadedness, vomiting, nausea, headache, confusion, 2) low potassium levels in your blood (hypokalemia) and 3) conditions related to swelling (edema). OLPRUVA™ contains salt (sodium), which can cause swelling from salt and water retention. Tell your doctor right away if you or your child get any of these symptoms. Your doctor may do certain blood tests to check for side effects during treatment with OLPRUVA™. If you have certain medical conditions such as heart, liver or kidney problems, are pregnant/planning to get pregnant or breast-feeding, your doctor will decide if OLPRUVA™ is right for you.

The most common side effects of OLPRUVA™ include absent or irregular menstrual periods, decreased appetite, body odor, bad taste or avoiding foods you ate prior to getting sick (taste aversion). These are not all of the possible side effects of OLPRUVA™. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

For additional Important Safety Information, see full [Prescribing Information](#), Patient Information and discuss with your doctor.

About [Acer Therapeutics Inc.](#)

Acer is a pharmaceutical company focused on the acquisition, development and commercialization of therapies for serious rare and life-threatening diseases with significant unmet medical needs. In the U.S., OLPRUVA™ (sodium phenylbutyrate) is approved for the treatment of urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). Acer is also advancing a pipeline of investigational product candidates for rare and life-threatening diseases, including: OLPRUVA™ (sodium phenylbutyrate) for treatment of various other inborn errors of metabolism, including Maple Syrup Urine Disease (MSUD); ACER-801 (osanetant) for treatment of induced Vasomotor Symptoms (iVMS) and Post-traumatic Stress Disorder (PTSD); EDSIVO™ (celiprolol) for treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation; and ACER-2820 (emetine), a host-directed therapy against a variety of viruses, including cytomegalovirus, Zika, dengue, Ebola and COVID-19. In March 2021, Acer entered into a Collaboration and License Agreement with Relief for development and commercialization of OLPRUVA™ in which Acer retains development and commercialization rights in the U.S., Canada, Brazil, Turkey, and Japan. For more information, visit www.acertx.com.

About [RELIEF THERAPEUTICS Holding SA](#)

RELIEF THERAPEUTICS Holding SA is a Swiss, commercial-stage, biopharmaceutical company focused on identification, development and commercialization of novel, patent protected products intended for the treatment of rare and ultra-rare diseases including metabolic disorders, pulmonary diseases and connective tissue disorders. Relief's diversified pipeline consists of assets with the potential to effectively address significant unmet medical needs, including PKU GOLIKE®, engineered with the proprietary Physiomimic™ technology, which is the first prolonged-release amino acid product commercialized for the dietary management of phenylketonuria (PKU). Relief has a collaboration and license agreement with Acer Therapeutics for the worldwide development and commercialization of ACER-001 (sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders

(UCDs) and maple syrup urine disease (MSUD). Relief also continues to develop aviptadil for several rare pulmonary indications. Further, Relief is in clinical development for APR-TD011, a differentiated acid oxidizing solution of hypochlorous acid intended for the treatment of epidermolysis bullosa (EB), a group of rare, genetic, life-threatening connective tissue disorders; APR-TD011 has been granted orphan drug designation by the U.S. FDA. Finally, Relief is commercializing several legacy products via licensing and distribution partners. RELIEF THERAPEUTICS Holding SA is listed on the SIX Swiss Exchange under the symbol RLF and quoted in the U.S. on OTCQB under the symbols RLTF and RLFTY. For more information, please visit www.relieftherapeutics.com. You may also follow Relief Therapeutics on [LinkedIn](#).

References

1. Ah Mew N, et al. Urea cycle disorders overview [updated June 22, 2017]. In: Adam MP, Ardinger HH, Pagon RA, et al, eds. GeneReviews® [Internet]. University of Washington; 1993-2022. Accessed March 20, 2022.
2. Häberle J, Boddaert N, Burlina A, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders. *Orphanet J Rare Dis.* 2012;7:32.
3. Gerstein MT, Markus AR, Gianattasio KZ, et al. Choosing between medical management and liver transplant in urea cycle disorders: a conceptual framework for parental treatment decision-making in rare disease. *J Inher Metab Dis.* 2020;43(3):438-458.
4. Peña-Quintana L, et al. Profile of sodium phenylbutyrate granules for the treatment of urea-cycle disorders: patient perspectives. *Patient Prefer Adherence.* 2017 Sep 6;11:1489-1496.

Acer Forward-Looking Statements

This press release contains “forward-looking statements” that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements, other than statements of historical facts, included in this press release regarding strategy, future operations, timelines for clinical study enrollment or regulatory actions, or otherwise, future financial position, future revenues, projected expenses, regulatory submissions, actions or approvals, cash position, liquidity, prospects, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to the potential for our investigational product candidates to safely and effectively treat diseases and to be approved for marketing; our ability to close upon and obtain the proceeds of any identified financing arrangements as well as to satisfy the ongoing conditions and requirements for maintaining the financing facilities and avoiding default or an accelerated payment requirement; the commercial or market opportunity and potential of OLPRUVA™ for the treatment of patients with UCDs, including the opportunity for approval in territories outside of the United States; the commercial or market opportunity of any of our product candidates in any target indication and any territory; our ability, in addition to the currently identified financings, to secure the additional capital necessary to fund our various product candidate development programs; the adequacy of our capital to support our future operations and our ability to successfully fund, initiate and complete clinical trials and regulatory submissions for OLPRUVA™ in MSUD, ACER-801, EDSIVO™ or our other investigational product candidates; the ability to protect our intellectual property rights; our strategy and business focus; and the development, expected timeline and commercial potential of any of our product candidates. Our pipeline product candidates are under investigation, their safety and efficacy have not been established and there is no guarantee that any of our investigational products in development will receive health authority approval or become commercially available for the uses being investigated. We may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Such statements are based on

management's current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation, risks and uncertainties associated with the ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the ability to launch successfully and sustain commercial viability of OLPRUVA™ for the treatment of patients with UCDs in the United States, the availability of sufficient resources to fund our various product candidate development programs and to meet our business objectives and operational requirements, the fact that the results of earlier studies and trials may not be predictive of future clinical trial results, the protection and market exclusivity provided by our intellectual property, risks related to the drug development and the regulatory approval process, including the timing and requirements of regulatory actions, and the impact of competitive products and technological changes. We disclaim any intent or obligation to update these forward-looking statements to reflect events or circumstances that exist after the date on which they were made. You should review additional disclosures we make in our filings with the Securities and Exchange Commission, including our Annual Report on Form 10-K and Quarterly Reports on Form 10-Q. You may access these documents for no charge at <http://www.sec.gov>.

Relief Forward-Looking Statements

This communication expressly or implicitly contains certain forward-looking statements concerning RELIEF THERAPEUTICS Holding SA and its businesses. Such statements involve certain known and unknown risks, uncertainties and other factors, including (i) whether RELIEF THERAPEUTICS Holding SA will submit an application for approval of ACER-001 in Europe and the timing of filing such application, (ii) whether any such application submitted to European authorities seeking marketing authorization for ACER-001 for the treatment of patients in Europe with UCDs will be approved, and (iii) those other risks, uncertainties and factors described in RELIEF THERAPEUTICS Holding SA's press releases and filings with the SIX Swiss Exchange and the U.S. Securities and Exchange Commission, all of which could cause the actual results, financial condition, performance or achievements of RELIEF THERAPEUTICS Holding SA to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. RELIEF THERAPEUTICS Holding SA is providing this communication as of this date and does not undertake to update any forward-looking statements contained herein as a result of new information, future events or otherwise.

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