



## Ad hoc announcement pursuant to Art. 53 LR

### Acer Therapeutics and Relief Therapeutics Announce Submission of a New Drug Application to the U.S. FDA for ACER-001 for Treatment of Urea Cycle Disorders

**NEWTON, MA and GENEVA, SWITZERLAND** – August 9, 2021 – Acer Therapeutics Inc. (Nasdaq: ACER) (“**Acer**”), a pharmaceutical company focused on the acquisition, development and commercialization of therapies for serious rare and life-threatening diseases with significant unmet medical needs, and RELIEF THERAPEUTICS Holding AG (SIX: RLF, OTCQB: RLFTF) (“**Relief**”), a biopharmaceutical company seeking to provide patients therapeutic relief from serious diseases with high unmet need, today announced the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for ACER-001 (sodium phenylbutyrate) for the treatment of Urea Cycle Disorders (UCDs). ACER-001 is a nitrogen-binding agent in development for use as adjunctive therapy in the chronic management of patients with UCDs involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS).

Based on standard FDA review timelines, Acer expects to receive notification from FDA on the potential acceptance of the NDA for filing within 60 days of submission and subsequent substantive review.

The 505(b)(2) NDA submission is supported by results from two previously announced bioequivalence (BE) trials in which ACER-001 showed similar relative bioavailability for both phenylbutyrate (PBA) and phenylacetate (PAA), the active metabolite of sodium phenylbutyrate, compared to BUPHENYL® (sodium phenylbutyrate). Acer has also received an Agreement Letter from FDA in response to the Company’s initial Pediatric Study Plan (iPSP) which outlines an agreed upon approach that addresses the needs of pediatric patients with UCDs.

“The submission of our NDA for ACER-001 marks an important step toward bringing this potential alternative treatment option to patients with UCDs,” said **Chris Schelling, Chief Executive Officer and Founder of Acer**. “We look forward to working with the FDA through the review process and will continue advancing our preparations for a potential launch of ACER-001, while also assisting Relief toward regulatory submissions in Europe.”

**Jack Weinstein, Chief Financial Officer and Treasurer of Relief Therapeutics**, added, “We are excited about the progress made to date in support of a potential regulatory approval of ACER-001 for UCDs in the U.S. With the NDA now submitted, we will continue our efforts to back ACER-001’s development in Europe by targeting submission of a Marketing Authorization Application (MAA) for the treatment of UCDs in Europe by the end of 2021.”

ACER-001 is an investigational product candidate which has not been approved by FDA or the European Medicines Agency (EMA). There is no guarantee that this product candidate will be accepted for substantive review, or if accepted, receive regulatory authority approval in any territory, or become commercially available for the indications under investigation.

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### ABOUT UCDS

UCDs are a group of disorders caused by genetic mutations that result in a deficiency in one of the six enzymes that catalyze the urea cycle, which can lead to an excess accumulation of ammonia in the bloodstream, a condition known as hyperammonemia. Acute hyperammonemia can cause lethargy, somnolence, coma, and multi-organ failure, while chronic hyperammonemia can lead to headaches, confusion, lethargy, failure to thrive, behavioral changes, and learning and cognitive deficits. Common symptoms of both acute and chronic hyperammonemia also include seizures and psychiatric symptoms.<sup>1,2</sup> The current treatment of UCDS consists of dietary management to limit ammonia production in conjunction with medications that provide alternative pathways for the removal of ammonia from the bloodstream. Some patients may also require individual branched-chain amino acid supplementation.

Current medical treatments for UCDS include nitrogen scavengers, RAVICTI® and BUPHENYL®, in which the active pharmaceutical ingredients are glycerol phenylbutyrate (GPB) and sodium phenylbutyrate, respectively. According to a 2016 study by Shchelochkov et al., published in *Molecular Genetics and Metabolism Reports*, while nitrogen scavenging medications have been shown to be effective in helping to manage ammonia levels in some patients with UCDS, non-compliance with treatment is common. Reasons referenced for non-compliance associated with some available medications include unpleasant taste, frequency with which medication must be taken, required number of pills, and the high cost of the medication.<sup>3</sup>

### ABOUT ACER-001

ACER-001 (sodium phenylbutyrate) is being developed for the treatment of various inborn errors of metabolism, including UCDS and MSUD. ACER-001 is a nitrogen-binding agent in development for use as adjunctive therapy in the chronic management of patients with UCDS involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). The formulation is a multi-particulate dosage formulation for oral administration consisting of a core center, a layer of active drug, and a taste-masked coating designed to avoid the bitter taste in the mouth while quickly dissolving in the low pH of the stomach. ACER-001's taste-masked formulation is aimed to improve the palatability of sodium phenylbutyrate. Acer is also being developed for Maple Syrup Urine Disease (MSUD) and has been granted orphan drug designation by the FDA for the MSUD indication. ACER-001 is an investigational product candidate which has not been approved by FDA or the European Medicines Agency (EMA).

### 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. Acer's pipeline includes four programs: ACER-001 (sodium phenylbutyrate) for treatment of various inborn errors of metabolism, including urea cycle disorders (UCDs) and Maple Syrup Urine Disease (MSUD); EDSIVO™ (celiprolol) for treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation; ACER-801 (osonetant) for treatment of induced Vasomotor Symptoms (iVMS); and ACER-2820 (emetine), a host-directed therapy against a variety of infectious diseases, including COVID-19. Each of Acer's product candidates is believed to present a comparatively de-risked profile, having one or more of a favorable

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safety profile, clinical proof-of-concept data, mechanistic differentiation and/or accelerated paths for development through specific programs and procedures established by the FDA. In March 2021, Acer entered into a Collaboration and License Agreement with Relief Therapeutics for development and commercialization of ACER-001. For more information, visit [www.acertx.com](http://www.acertx.com).

### ABOUT RELIEF THERAPEUTICS HOLDING AG

Relief focuses primarily on clinical-stage programs based on molecules with a history of clinical testing and use in human patients or a strong scientific rationale. Relief's lead drug candidate RLF-100™ (aviptadil), a synthetic form of Vasoactive Intestinal Peptide (VIP), is in late-stage clinical testing in the U.S. for the treatment of respiratory deficiency due to COVID-19. As part of its pipeline diversification strategy, in March 2021, Relief entered into a Collaboration and License Agreement with Acer Therapeutics for development and commercialization of ACER-001. ACER-001 is a taste-masked and immediate release proprietary powder formulation of sodium phenylbutyrate (NaPB) for the treatment of Urea Cycle Disorders and Maple Syrup Urine Disease. In addition, Relief's recently completed acquisition of APR Applied Pharma Research SA brings a diverse pipeline of marketed and development-stage programs.

RELIEF THERAPEUTICS Holding AG is listed on the SIX Swiss Exchange under the symbol RLF and quoted in the U.S. on OTCQB under the symbol RLFTF.

For more information, visit [www.relieftherapeutics.com](http://www.relieftherapeutics.com).

Follow Relief on [LinkedIn](#).

### REFERENCES

1. Ah Mew N, et al. Urea cycle disorders overview. Gene Reviews. Seattle, Washington: University of Washington, Seattle; 1993.
2. Häberle J, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders. Orphanet Journal of Rare Diseases. 2012;7(32).
3. Shchelochkov OA, et al. Barriers to drug adherence in the treatment of urea cycle disorders: Assessment of patient, caregiver and provider perspectives. Mol Genet Metab. 2016;8:43-47.

### 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, 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 product candidates to safely and effectively treat diseases and to be approved for marketing; the commercial or market opportunity of any of our product candidates in any target indication and any territory;



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our ability 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; 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. 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 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 Quarterly Reports on Form 10-Q and our Annual Report on Form 10-K. 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 AG and its businesses. The results reported herein may or may not be indicative of the results of future and larger clinical trials for ACER-001 for the treatment of UCDs and MSUD, nor whether the ongoing clinical trials of Relief's lead compound, RLF-100™ (aviptadil) in advanced clinical development to treat respiratory deficiency due to COVID-19, will be successful. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of RELIEF THERAPEUTICS Holding AG to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. RELIEF THERAPEUTICS Holding AG 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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