



# Pharming Group N.V. and HAEi International Patient Organization announce partnership with Inceptua Medicines Access for *"HAEi Global Access Program"*

Allows hereditary angioedema (HAE) patients in all countries to gain access to RUCONEST<sup>®</sup> via Medicines Access Program

Leiden, the Netherlands and LUXEMBOURG – 26 Sept 2017 - Pharming Group N.V in association with HAEi, the international umbrella organization for the world's Hereditary Angioedema (HAE) patient groups, announce the appointment of Inceptua Medicines Access as their new distribution partner for the "HAEi Global Access Program" (HAEi GAP) enabling patients in all countries where Pharming's product RUCONEST<sup>®</sup> is not commercially available to gain access to the drug through an ethical and regulatory compliant mechanism. It is the only known program of this type which has been initiated through a patient group.

The program is the only Global Access Program in hereditary angioedema (HAE), a very rare and potentially life-threatening genetic condition that occurs in about 1 in 10,000 (1) to 1 in 50,000 people (2) HAE symptoms include attacks of swelling in various body parts, including the hands, feet, face, intestinal walls and airway. Swelling in the airway is particularly dangerous and can lead to death by asphyxiation. (2) There is currently no cure for HAE, but certain treatments exist to prevent and ease attacks. These treatments are not licensed or available in all countries worldwide, leaving patients unable to prepare for and treat attacks.

RUCONEST<sup>®</sup> is the first treatment to be made available through the HAEi GAP program in countries where it is not commercially available. RUCONEST<sup>®</sup> is a recombinant human C1- inhibitor, approved by the European Medicines Agency (EMA) and US Food and Drug Administration (FDA) for the treatment of acute attacks of hereditary angioedema (HAE) (3,4)

Physicians wishing to request RUCONEST<sup>®</sup> for their patients through the HAEi GAP program should contact <u>HAEiGAP@inceptua.com</u> or alternatively ring +44 20 3910 7670. Please note that direct patient inquiries cannot be handled.

"HAEi exists to support patients with HAE gain access to HAE therapies to improve their quality of life and potentially be life-saving in the case of a laryngeal attack," commented Henrik Balle Boysen, HAEi Executive Director. "Through this partnership with Pharming Group N.V. and Inceptua Medicines Access, physicians, who may otherwise have no access to modern HAE therapies, have the opportunity to request RUCONEST for their patients, effective immediately."

"We are delighted to announce, in association with HAEi, that Inceptua Medicines Access are our new trusted distribution partner, offering a very tailored solution, providing navigation support to physicians in an ethical and regulatory compliant way to gain access to this urgently-required treatment for their patients," said Paul Janssen, Pharming Group N.V., Vice-President Commercial Operations Europe and ROW.

Mark Corbett, Executive Vice-President, Inceptua Medicines Access commented: "We are providing a transformational approach to medicines access, we will strive to provide unrivalled service in





navigating access to RUCONEST<sup>®</sup> for the treatment of patients in need. We are delighted to work with both HAEi and Pharming in delivering what is the only known Medicines Access Program instigated by a patient organization. We will navigate access to this medicine with agility, quality and regulatory compliance, ultimately to help the treatment of patients in need."

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Contacts for media inquiries or further information: Pharming Group N.V.

Sijmen de Vries, CEO, Tel: +31 71 524 7400 Robin Wright, CFO, Tel: +31 71 524 7400

FTI Consulting, London, UK:

Julia Phillips/ Victoria Foster Mitchell, T: +44 203 727 1136

LifeSpring Life Sciences Communication, Amsterdam, The Netherlands:

Leon Melens, Tel: +31 6 53 81 64 27

Inceptua

Nicola Lilley Havas SO nicola@sowhatglobal.com +44 7983 128 712

Mark Corbett **Executive Vice President, Inceptua Medicines Access** <u>Medicinesaccess@inceptua.com</u> +44 207 907 9200

# About HAE

Hereditary Angioedema (HAE) is a rare genetic disorder. It is characterized by spontaneous and recurrent episodes of swelling (edema attacks) of the skin in different parts of the body, as well as in the airways and internal organs (4,5). Edema of the skin usually affects the extremities, the face, and the genitals. Patients suffering from this kind of edema often withdraw from their social lives because of the disfiguration, discomfort and pain these symptoms may cause. (6) Almost all HAE patients suffer from bouts of severe abdominal pain, nausea, vomiting and diarrhoea caused by swelling of the intestinal wall. (7)

Edema of the throat, nose or tongue can be particularly dangerous as this can lead to obstruction of the airway passages and be potentially life threatening (2). Although there is currently no known cure for HAE, it is possible to treat the symptoms associated with edema attacks. HAE affects about 1 in 10,000 to 1 in 50,000 people worldwide (1,2) experts believe that a lot of patients are still seeking the right diagnosis: although HAE is (in principle) easy to diagnose, it is frequently identified very late or not discovered at all. The reason HAE is often misdiagnosed is because the symptoms are similar to those of many other common conditions such as allergies or appendicitis (5) by the time it is diagnosed correctly, the patient has often been through a long-lasting ordeal.





## About Global Access Programs

Global Access Programs provide biopharmaceutical companies with a way to allow ethical access to their pre-license/unlicensed medicines to help patients with unmet medical needs. Access is provided in response to physician requests, in a fully compliant manner, where no alternative treatment options are available.

## About HAEi

HAEi is the international umbrella organization for the world's Hereditary Angioedema (HAE) patient groups. HAEi is a global non-profit **network of patient associations** and dedicated to raising awareness of C1 inhibitor deficiencies around the world. HAEi strives to improve the time to diagnosis and facilitate access to and reimbursement of life saving HAE therapies, which will enable lifelong health for all patients – no matter where they live.

For more information, please visit: <u>www.haei.org</u>

## About RUCONEST®

## **US INDICATION**

RUCONEST<sup>®</sup> (C1 esterase inhibitor [recombinant]) is indicated for the treatment of acute attacks in adult and adolescent patients with hereditary angioedema (HAE). Effectiveness in clinical studies was not established in HAE patients with laryngeal attacks.

#### IMPORTANT SAFETY INFORMATION

- RUCONEST<sup>®</sup> (C1 esterase inhibitor [recombinant]) is contraindicated in:
  - Patients with a history of allergy to rabbits or rabbit-derived products.
  - Patients with a history of life-threatening immediate hypersensitivity reactions to C1 esterase inhibitor preparations, including anaphylaxis.
- Hypersensitivity: Severe hypersensitivity reactions may occur. Should symptoms occur, discontinue RUCONEST and administer appropriate treatment. Because hypersensitivity reactions may have symptoms similar to HAE attacks, treatment methods should be carefully considered.
- Thromboembolic Events: Serious arterial and venous thromboembolic (TE) events have been reported at the recommended dose of plasma-derived C1 esterase inhibitor products in patients with risk factors. Risk factors may include the presence of an indwelling venous catheter/access device, prior history of thrombosis, underlying atherosclerosis, use of oral contraceptives or certain androgens, morbid obesity, and immobility. Monitor patients with known risk factors for TE events during and after RUCONEST administration.
- Intravenous Use: RUCONEST is for intravenous use after reconstitution only. No more than 2 doses should be administered within a 24-hour period.
- **Pregnancy and Nursing**: RUCONEST has not been studied in pregnant women; therefore, it should only be used during pregnancy if clearly needed. Advise patients to notify their physician if they are breastfeeding or plan to breastfeed.





- Adverse reactions: The serious adverse reaction in clinical studies of RUCONEST was anaphylaxis.
- Common adverse reactions: The most common adverse reactions (incidence ≥2%) were headache, nausea, and diarrhea.

Please see Full Prescribing Information for RUCONEST<sup>®</sup> as applicable for various jurisdictions: <u>FDA: RUCONEST<sup>®1</sup> / EMA: RUCONEST<sup>®2</sup></u>

# About Pharming Group N.V.

Pharming is a specialty pharmaceutical company developing innovative products for the safe, effective treatment of rare diseases and unmet medical needs. Pharming's lead product, RUCONEST® (conestat alfa) is a recombinant human C1 esterase inhibitor approved for the treatment of acute Hereditary Angioedema ("HAE") attacks in patients in Europe, the US, Israel and South Korea. The product is available on a named-patient basis in other territories where it has not yet obtained marketing authorization.

RUCONEST<sup>®</sup> is commercialized by Pharming in Algeria, Andorra, Austria, Bahrain, Belgium, France, Germany, Ireland, Jordan, Kuwait, Lebanon, Luxembourg, Morocco, the Netherlands, Oman, Portugal, Qatar, Syria, Spain, Switzerland, Tunisia, the United Arab Emirates, the United Kingdom, the United States of America and Yemen.

RUCONEST<sup>®</sup> is distributed by Swedish Orphan Biovitrum AB (publ) (SS: SOBI) in the other EU countries, and in Azerbaijan, Belarus, Georgia, Iceland, Kazakhstan, Liechtenstein, Norway, Russia, Serbia and Ukraine.

RUCONEST<sup>®</sup> is distributed in Argentina, Colombia, Costa Rica, the Dominican Republic, Panama, and Venezuela by Cytobioteck, in South Korea by HyupJin Corporation and in Israel by Megapharm.

RUCONEST<sup>®</sup> is also being investigated in a Phase II clinical trial for the treatment of HAE in young children (2-13 years of age) and evaluated for various additional follow-on indications.

Pharming's technology platform includes a unique, GMP-compliant, validated process for the production of pure recombinant human proteins that has proven capable of producing

industrial quantities of high quality recombinant human proteins in a more economical and less immunogenetic way compared with current cell-line based methods. Leads for enzyme

<sup>&</sup>lt;sup>1</sup><u>https://www.fda.gov/downloads/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/LicensedProductsBLAs/Fr</u> actionatedPlasmaProducts/UCM405634.pdf

<sup>&</sup>lt;sup>2</sup><u>http://www.ema.europa.eu/docs/en\_GB/document\_library/EPAR\_-</u> Product\_Information/human/001223/WC500098542.pdf





replacement therapy ("ERT") for Pompé and Fabry's diseases are being optimized at present, with additional programs not involving ERT also being explored at an early stage at present.

Pharming has a long-term partnership with the China State Institute of Pharmaceutical Industry ("CSIPI"), a Sinopharm company, for joint global development of new products, starting with recombinant human Factor VIII for the treatment of Haemophilia A. Pre-clinical development and manufacturing will take place to global standards at CSIPI and are funded by CSIPI. Clinical development will be shared between the partners with each partner taking the costs for their territories under the partnership.

Pharming has declared that the Netherlands is its "Home Member State" pursuant to the amended article 5:25a paragraph 2 of the Dutch Financial Supervision Act.

Additional information is available on the Pharming website: www.pharming.com

# Forward-looking Statements

This press release of Pharming Group N.V. and its subsidiaries ("Pharming", the "Company" or the "Group") may contain forward-looking statements including without limitation those regarding Pharming's financial projections, market expectations, developments, partnerships, plans, strategies and capital expenditures.

The Company cautions that such forward-looking statements may involve certain risks and uncertainties, and actual results may differ. Risks and uncertainties include without limitation the effect of competitive, political and economic factors, legal claims, the Company's ability to protect intellectual property, fluctuations in exchange and interest rates, changes in taxation laws or rates, changes in legislation or accountancy practices and the Company's ability to identify, develop and successfully commercialize new products, markets or technologies.

As a result, the Company's actual performance, position and financial results and statements may differ materially from the plans, goals and expectations set forth in such forward-looking statements. The Company assumes no obligation to update any forward-looking statements or information, which should be taken as of their respective dates of issue, unless required by laws or regulations.

# About Inceptua Medicines Access

Inceptua Medicines Access is a transformative global medicines access provider designed to rise to the evolving challenges of the environment offering unsurpassed understanding and insight into the needs of all stakeholders. Inceptua Medicines Access offers unrivalled delivery and implementation of services, and cutting-edge solutions. We are leading experts working in partnership with the biopharmaceutical industry, regulators and patient organizations to deliver urgently-required medicines to healthcare professionals treating patients in need.

Inceptua Medicines Access aims to transform the future of access to pre-approval and unlicensed medicines, powered by understanding and insight into the needs of biopharmaceutical companies and healthcare organizations, and delivered with compassion for patients and the medicines they need.

Inceptua Medicines Access is a business unit of Multipharma Group, a dynamic, enterprising, and future-oriented service provider operating within the clinical trial and unlicensed medicines supply chain at an international level.





Our multinational team provide connections to an extensive global network, offering our clients customized solutions ranging from strategic planning and sourcing, clinical manufacturing and global depot solutions, right through to early access to unlicensed medicines.

We are committed to safety, and reliably supporting our clients every step of the way, leaving them safe in the knowledge that every potential obstacle has been considered, and every eventuality planned for.

Our worldwide presence enables us to fulfil these services for the duration of trials, and our expert team work closely with our customers to ensure they receive full support throughout the lifecycle of their project.

For more information, please visit our website at <u>www.inceptua.com</u>.

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