REGENX Biosciences and ESTEVE enter into license agreement for rare lysosomal storage disorder

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WASHINGTON, DC and BARCELONA, SPAIN February 28, 2014 – REGENX Biosciences, LLC (REGENX) and Laboratorios Dr. Esteve, S.A. (ESTEVE) announce that they have entered into an agreement enabling the development and commercialization of products to treat mucopolysaccharidosis type IIIA (MPSIIIA or Sanfilippo syndrome Type A) using **NAV** rAAV9.

Under the terms of the Agreement, REGENX granted ESTEVE a non-exclusive worldwide license, with rights to sublicense, to REGENX's **NAV** rAAV9 vectors for treatment of MPS IIIA in humans. In return for these rights, REGENX receives payments in the form of an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAV9.

"We believe this license agreement will further advance the development of **NAV**-based gene delivery treatments for patients with MPS IIIA," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to further our mission of enabling the development of successful new AAV therapeutics by collaborating with the ESTEVE team."

"We are happy to be working with REGENX and believe the signing of this agreement enables ESTEVE to advance the development of our gene therapeutic for Sanfilippo A towards clinical trials," said Albert Esteve, CEO of ESTEVE. "We share the same mission as ReGenX - the development of innovative products to meet patient needs - and that is why this is one of our highest priority projects today."

About MPS III A (Sanfilippo syndrome Type A)

Sanfilippo syndrome is a devastating disease that leads to progressive and significant deterioration in mental status of children who rarely live beyond their twenties. The Sanfilippo syndrome Type IIIA is a lysosomal storage disease caused by the loss of the activity of the enzyme sulfamidase. It affects approximately 1 in 100,000 births and is often diagnosed only once symptoms have begun to appear.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary **NAV** vector technology platform, for a range of severe diseases with serious unmet needs. **NAV** vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Lysogene, and Audentes Therapeutics. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding REGENX, please visit www.regenxbio.com.

About ESTEVE

ESTEVE (www.esteve.com) is a leading pharmaceutical chemical group based in Barcelona, Spain. Since it was founded in 1929, ESTEVE has been firmly committed to excellence in healthcare, dedicating efforts to innovative R&D of new medicines for unmet medical needs and focusing on high science and evidence-based research. ESTEVE has a strong partnership approach to drug discovery, development and commercialization. The company works both independently and in collaboration to bring new, differentiated best-in-class treatments to patients who need them. The company currently employs 2,300 professionals and has subsidiaries and production facilities in several European countries, USA,

China and Mexico.

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ESTEVE

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