Media Contact:
Sarah Freeman
Phone: +1 215-510-4758
sfreem21@its.jnj.com

Investor Relations:
Christopher DelOrefice
Office: +1 732-524-2955

Jennifer McIntyre
Office: +1 732-524-3922

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Interim Six-Month Data of RPGR Gene Therapy Shows Significant Vision Improvement in Patients Living with X-Linked Retinitis Pigmentosa

First data to read out from the Janssen and MeiraGTx collaboration

Johnson & Johnson to review interim Phase 1/2 clinical trial findings in pre-recorded webcast

Company anticipates progressing the program to Phase 3

RARITAN, NJ, July 17, 2020 – The Janssen Pharmaceutical Companies of Johnson & Johnson announced today six-month data from the ongoing Phase 1/2 trial (NCT03252847) of its investigational gene therapy for the treatment of inherited retinal disease X-linked retinitis pigmentosa (XLRP). The interim data showed that low and intermediate doses of the investigational adeno-associated virus retinitis pigmentosa GTPase regulator (AAV-RPGR) were generally well-tolerated and indicated significant improvement in vision. Initial data on the novel AAV-RPGR asset, jointly developed with MeiraGTx Holdings plc, will be available today as a late-breaker, pre-recorded oral presentation at the American Society of Retina Specialists (ASRS) 2020 Virtual Annual Meeting.

In patients with XLRP, the photoreceptors in the eye that are responsible for converting light into signals that are sent to the brain function poorly, leading to degeneration of the retina

and legal blindness in adulthood. The companies' AAV-RPGR gene therapy is being investigated to treat the most common and severe forms of XLRP caused by mutations in the RPGR gene by preserving and improving vision and slowing retinal degeneration. Currently, there are no approved treatments for this condition.

"There is an urgent need to deliver a transformational therapy for people living with XLRP who experience progressive visual loss from childhood with eventual blindness in early adulthood," said Michel Michaelides[i], B.Sc., M.B., B.S., M.D. (Res), FRCOphth, FACS, trial investigator, Consultant Ophthalmologist, Moorfields Eye Hospital, Professor of Ophthalmology, University College London. "We have learned valuable safety and efficacy information from this Phase 1/2 trial and look forward to applying those learnings in our next phase of study."

The ongoing Phase 1/2 clinical trial consists of three phases: dose-escalation, dose-confirmation and dose-expansion. In the dose-escalation phase (n=10), adults were administered low, intermediate, or high dose AAV-RPGR. The primary endpoint of the trial is safety, with secondary endpoints assessing changes in visual function at pre-specified timepoints post-treatment. Baseline values were determined in triplicate.

In the dose escalation phase, at six months, the low (n=3) and intermediate (n=4) dose cohorts demonstrated significant improvement from baseline in retinal sensitivity after treatment. Importantly, these improvements were evident when assessed with two perimetry approaches (static perimetry and microperimetry) and three analysis metrics (mean retinal sensitivity, central 30° hill-of-vision volumetric measure [V30], and pointwise comparison). These interim results from the Phase 1/2 trial participants suggest that the findings are significant:

- Significant differences in mean retinal sensitivity were observed between treated and untreated eyes in the intermediate dose cohort: 1.02 decibel (dB); (90% CI: 0.75, 1.31).
- Significant differences were observed in central visual field progression rate (V30) between treated and untreated eyes in the low: 1.10 dB-sr (steradian)/year; (90% CI: 0.10, 2.10) and intermediate: 1.26 dB-sr/year; (90% CI: 0.65, 1.86) dose cohorts[ii].

- Efficacy signals were observed at first post-treatment assessments at three months with improvements generally sustained or increased at six months.

Perimetry is a sensitive standard-of-care measure of retinal function that reproducibly determines retinal sensitivity both cross-sectionally and longitudinally, thereby accurately defining disease progression over time.

Safety data obtained to date has ocular and systemic safety profiles that are anticipated and manageable. The most common adverse events were related to the surgical procedure, transient and resolved without intervention. In the high dose cohort (n=3), inflammation was evident in two of three adults and measures of visual function were not improved.

"These findings demonstrate the potential of our investigational AAV-RPGR gene therapy not just to preserve, but improve vision for people living with XLRP," said James List, M.D., Ph.D., Global Therapeutic Area Head, Cardiovascular & Metabolism, Janssen Research & Development, LLC. "We are encouraged by the data we have seen to date, and look forward to sharing future read-outs and advancing our clinical development program."

"With patient needs setting our priorities, Janssen is committed to investing in our retinal portfolio and working tirelessly to bring transformational therapies to patients worldwide," said Mathai Mammen, M.D., Ph.D., Global Head, Janssen Research & Development, Johnson & Johnson. "These new data highlight our progress to advance this clinical program and support further study of our investigational gene therapy."

Johnson & Johnson's pre-recorded webcast, which reviews interim data from the Phase 1/2 RPGR clinical trial and Janssen's retinal portfolio strategy, features James List, M.D., Ph.D., Global Therapeutic Area Head, Cardiovascular & Metabolism, Janssen Research & Development, LLC, and can be accessed on the Johnson & Johnson website at www.investor.jnj.com by clicking on "Webcasts/Presentations". The webcast is approximately ten minutes and will be available through the end of August 2020. ASRS will also host a live Q&A session on July 25, 2020 from 11:55AM–12:05PM (ET) for registered meeting participants.

About the Phase 1/2 RPGR Trial (MGT009) and AAV-RPGR

MGT009 (NCT03252847) is an open-label, multi-center, dose-escalation trial, which enrolled

patients aged five years and older with XLRP caused by mutations in RPGR at multiple sites in the United States and United Kingdom. The primary endpoint was safety and tolerability; secondary endpoints assessed retinal sensitivity, visual function, functional vision and quality of life measurements.

AAV-RPGR was delivered via subretinal injection targeting the central retina in the eye that was more affected at baseline. The patient's other eye served as an untreated control. Multiple retinotomies were permitted to enable coverage of the largest possible area of rescuable retina. Perimetry was performed using Octopus 900 full-field static perimetry and MAIA fundus-guided microperimetry, and was conducted at baseline, three, six, nine and 12 months to assess baseline retinal function and change over time. Patients were required to have evidence of relative preservation of retinal structure at the macula and be able to undertake age-appropriate clinical assessments. For more information, visit: https://clinicaltrials.gov/ct2/show/NCT03252847?.

The European Medicines Agency (EMA) granted PRIME (PRIority MEdicines) and Advanced Therapy Medicinal Product (ATMP) designations to the company's RPGR gene therapy to increase interactions and optimize development plans based on data from the ongoing Phase 1/2 clinical trial. The novel AAV-RPGR therapy also received Fast Track designation from the U.S. Food and Drug Administration (FDA) and Orphan designations from the FDA and the EMA.

About the Janssen and MeiraGTx Strategic Collaboration

In January 2019, Janssen entered into a worldwide collaboration and license agreement with MeiraGTx Holdings plc, a clinical-stage gene therapy company, to develop, manufacture and commercialize its clinical-stage inherited retinal disease portfolio. AAV-RPGR gene therapy is being developed under this collaboration and license agreement. In addition to forming a research collaboration to explore new targets for other inherited retinal diseases, Janssen is working with MeiraGTx to build core capabilities in viral vector design, optimization and manufacturing.

About Janssen's Retinal Portfolio

We are translating our understanding of the biology underlying retinal diseases to develop needed therapies that preserve and enhance vision. Janssen's clinical-stage retinal portfolio includes leading product candidates for inherited retinal diseases XLRP and achromatopsia.

Janssen is also expanding into more common eye diseases, including wet age-related macular degeneration, diabetic retinopathy and diabetic macular edema.

About the Janssen Pharmaceutical Companies of Johnson & Johnson

At Janssen, we're creating a future where disease is a thing of the past. We're the Pharmaceutical Companies of Johnson & Johnson, working tirelessly to make that future a reality for patients everywhere by fighting sickness with science, improving access with ingenuity and healing hopelessness with heart. We focus on areas of medicine where we can make the biggest difference: Cardiovascular & Metabolism, Immunology, Infectious Diseases & Vaccines, Neuroscience, Oncology and Pulmonary Hypertension. Learn more at www.janssen.com. Follow us at www.twitter.com/JanssenGlobal. Janssen Research & Development, LLC is part of the Janssen Pharmaceutical Companies of Johnson & Johnson.

Cautions Concerning Forward-Looking Statements

This press release and webcast contain "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding the potential benefits of the collaboration and license agreement with MeiraGTx Holdings plc. The reader is cautioned not to rely on these forward-looking statements. These statements are based on current expectations of future events. If underlying assumptions prove inaccurate or known or unknown risks or uncertainties materialize, actual results could vary materially from the expectations and projections of Janssen Research & Development, LLC, any of the other Janssen Pharmaceutical Companies and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in product research and development, including the uncertainty of clinical success and of obtaining regulatory approvals; uncertainty of commercial success; manufacturing difficulties and delays; competition, including technological advances, new products and patents attained by competitors; challenges to patents; product efficacy or safety concerns resulting in product recalls or regulatory action; changes in behavior and spending patterns of purchasers of health care products and services; changes to applicable laws and regulations, including global health care reforms; and trends toward health care cost containment. A further list and descriptions of these risks, uncertainties and other factors can be found in Johnson & Johnson's Annual Report on Form 10-K for the fiscal year ended December 29, 2019, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and in the company's subsequent Quarterly

Reports on Form 10-Q, and other filings with the Securities and Exchange Commission. Copies of these filings are available online at www.sec.gov, www.jnj.com or on request from Johnson & Johnson. Neither the Janssen Pharmaceutical Companies nor Johnson & Johnson undertakes to update any forward-looking statement as a result of new information or future events or developments.

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[i] *Dr. Michaelides is a scientific founder of and consultant to MeiraGTx.*
[ii] *Modeling includes all available data for nine adult participants excluding one participant with panuveitis in the low dose group.*