Save the Date

Round-Up Webinar Series: Expert Perspectives in Fabry Disease

Thursday 20th October 2022, 18:00–19:30 (BST)

Following the huge success of webinar one in the Round-Up Webinar Series, it is with great pleasure that our faculty invite you to join them for the second part of this Sanofi-sponsored webinar series examining expert perspectives in Fabry disease. In this webinar, **'Genetic underpinnings of Fabry disease and Fabry nephropathy'**, our faculty will provide evidence based updates on the impact of Fabry disease on patients' lives and the role of clinical genetics in the understanding of Fabry disease. Following these exciting discussions there will be an opportunity for Q&A with our expert faculty. Please see below for further information on our speakers and the agenda this webinar.

We look forward to seeing you there!

REGISTER FOR WEBINAR 2

Webinar 2: Genetic underpinnings of Fabry disease and Fabry nephropathy

Timing	Presentation	Speaker
5 minutes	**Introduction**	**Prof. Derralynn Hughes** Professor of Experimental Haematology and Clinical Director of Research and Innovation at the Royal Free London NHS Foundation Trust
20 minutes	**Impacts of clinical genetics in Fabry disease**	**Dr Paul Brennan** Consultant in Clinical Genetics, Northern Genetics Service at Newcastle upon Tyne Hospitals NHS Foundation Trust, and Clinical Senior Lecturer in Genetic Medicine, University of Newcastle upon Tyne, UK
10 minutes	**Presentation of a local case study**	**Dr Patrick Deegan** Senior Visiting Research Fellow and NHS Consultant in Metabolic and General Medicine, Cambridge University Hospitals NHS Foundation Trust, UK
20 minutes	**Understanding Fabry disease from the perspective of unexplained chronic kidney disease**	**Prof. John Sayer** Institute of Human Genetics, International Centre for Life, University of Newcastle upon Tyne, UK
10 minutes	**Presentation of a local case study**	**Dr Karolina Stepien** Consultant in Adult Inherited Metabolic Disorders at Salford Royal NHS Foundation Trust, UK
25 minutes	**Q&A and session close**	**All**

Please note, this webinar has been created in line with the UK prescribing indications for Fabrazyme®.
Sanofi does not recommend the use of any product outside of their approved indications.
Please consult your local prescribing information before prescribing

This Sanofi-sponsored event is intended for healthcare professionals only.

MAT-XU-2204015 (V1.0) | Date of preparation: October 2022

PRESCRIBING INFORMATION

Prescribing Information: Fabrazyme (agalsidase beta) 5mg/ 35mg powder for concentrate for solution for infusion Please refer to the Summary of Product Characteristics (SmPC) before prescribing. **Presentations:** Each vial contains 5mg or 35mg of agalsidase beta and excipients. Following reconstitution with water for injections each vial contains 5mg/ml agalsidase beta. **Indication:** Fabrazyme is indicated in adults, children and adolescents aged 8 years and older, for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency). **Dosage and administration:** The treatment should be supervised by a physician experienced in the management of patients with Fabry Disease or other inherited metabolic diseases. The recommended dose of Fabrazyme is 1mg/kg body weight administered once every 2 weeks as an intravenous infusion. Infusion of Fabrazyme at home may be considered for patients who are tolerating their infusions well. The decision to have a patient move to home infusion should be made after evaluation and recommendation by the treating physician. Patients experiencing adverse events during the home infusion need to immediately **stop the infusion process** and seek the attention of a healthcare professional. Subsequent infusions may need to occur in a clinical setting. Dose and infusion rate should remain constant while at home, and not be changed without supervision of a healthcare professional. Fabrazyme should be administered as an intravenous infusion. The initial infusion rate should be no more than 0.25 mg/min (15 mg/hour) to minimise the potential occurrence of infusion-associated reactions. After patient tolerance is established, the infusion rate may be increased gradually with subsequent infusions. The number of vials should be determined to be reconstituted based on the individual patient's weight. Each vial of Fabrazyme 35 mg has to be reconstituted with 7.2 ml water for injections and each vial of Fabrazyme 5 mg has to be reconstituted with 1.1 ml water for injections. The reconstituted solution should be slowly injected directly into the 0.9% sodium chloride solution for injection (not in any remaining airspace) to a final concentration between 0.05 mg/ml and 0.7 mg/ml. The total volume of sodium chloride 0.9% solution for infusion (between 50 and 500 ml) should be determined based on the individual dose. For doses lower than 35 mg a minimum of 50 ml should be used, for doses 35 to 70 mg a minimum of 100 ml should be used, for doses 70 to 100 mg a minimum of 250 ml should be used and for doses greater than 100 mg only 500 ml should be used. It is recommended to administer the diluted solution through an in-line low protein-binding 0.2 µm filter. Fabrazyme must not be mixed with other medicinal products in the same infusion. **Special populations:** No dose adjustment is necessary for children 8-16 years. The safety and efficacy of Fabrazyme in children 0-7 years and in patients older than 65 years have not been established and no dosage regimen can presently be recommended in these patients. No dose adjustment is necessary for patients with renal insufficiency. Studies in patients with hepatic insufficiency have not been performed. **Contraindications:** Life-threatening hypersensitivity (anaphylactic reaction) to the active substance or any of the excipients. **Warnings and Precautions:** *Immunogenicity:* The majority of patients are expected to develop IgG antibodies to agalsidase beta typically within 3 months of initiation of treatment. Over time, the majority of seropositive patients in clinical trials demonstrated either a downward trend in titres, tolerised or demonstrated a plateau. *Infusion-associated reactions (IAR):* Patients with antibodies to r-hαGAL have a greater potential to experience IARs. These patients should be treated with caution when re-administering agalsidase beta. Antibody status should be regularly monitored. In clinical trials, 67% of patients experienced at least one IAR. Patients experiencing mild or moderate infusion associated reactions have continued therapy after a reduction in the infusion rate (~0.15 mg/min; 10 mg/hr) and/or pre-treatment with antihistamines, paracetamol, ibuprofen and/or corticosteroids. The frequency of these reactions decreased over time. Antibody status should be regularly monitored. *Hypersensitivity:* As with any intravenous protein product, allergic-type hypersensitivity reactions are possible. If severe allergic or anaphylactic-type reactions occur, immediate discontinuation of the administration of Fabrazyme should be considered and appropriate treatment be initiated. The current medical standards for emergency treatment are to be observed. *Patients with advanced renal disease:* The effect of Fabrazyme treatment on the kidneys may be limited in these patients. *Fertility, pregnancy and lactation:* There are no adequate data from the use of agalsidase beta in pregnant women or its potential effect on fertility. Animal studies do not indicate direct or indirect harmful effects with respect to embryonal/foetal development. Fabrazyme should not be used during pregnancy unless clearly necessary. Agalsidase beta may be excreted in breast milk, it is recommended to stop breast-feeding when Fabrazyme is used. *Sodium:* This medicinal product contains less than 1 mmol sodium (23 mg) per vial that is to say essentially 'sodium-free'. *Traceability:* In order to improve the traceability of biological medicinal products, the name and the batch number of the administered medicinal product should be clearly recorded. **Interactions:** No interaction studies and no *in vitro* metabolism studies have been performed. Fabrazyme should not be administered with chloroquine, amiodarone, benoquin or gentamycin due to a theoretical risk of inhibition of intra-cellular α-galactosidase A activity. **Adverse effects:** *Very Common (≥1/10):* Headache, paraesthesia, nausea, vomiting, chills, pyrexia, and feeling cold. *Common (≥1/100 to <1/10):* nasopharyngitis, dizziness, somnolence, hypoaesthesia, burning sensation, lethargy, syncope, lacrimation increased, tinnitus, vertigo, tachycardia, palpitations, bradycardia, flushing, hypertension, pallor, hypotension, hot flush, dyspnoea, nasal congestion, throat tightness, wheezing, cough, dyspnoea exacerbated, abdominal pain, abdominal pain upper, abdominal discomfort, stomach discomfort, hypoaesthesia oral, diarrhoea, pruritus, urticaria, rash, erythema, pruritus generalised, angioneurotic oedema, swelling face, rash maculopapular, pain in extremity, myalgia, back pain, muscle spasms, arthralgia, muscle tightness, musculoskeletal stiffness, fatigue, chest discomfort, feeling hot, oedema peripheral, pain, asthenia, chest pain, face oedema, hyperthermia. **Legal category:** POM. **Marketing authorisation holder:** Sanofi Genzyme, 410 Thames Valley Park Drive, Reading, Berkshire, RG6 1PT, UK. **Marketing authorisation numbers and UK List prices:** Fabrazyme 5mg powder for concentrate for solution for infusion x 1 vial: £315.08, PLGB 04425/0768. Fabrazyme 35mg powder for concentrate for solution for infusion x 1 vial: £2196.59, PLGB 04425/0767. **For more information please contact:** Medical Information, Sanofi, 410 Thames Valley Park Drive, Reading, Berkshire, RG6 1PT, UK. uk-medicalinformation@sanofi.com **Date of Preparation:** July 2021. **MAT-GB-2103132(v1.0)**

Adverse events should be reported. Reporting forms and information can be found at www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. Adverse events should also be reported to Sanofi drug safety department on Tel: 0800 0902 314. Alternatively, send via email to UK-drugsafety@sanofi.com

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In clinical trials with classic patients, the infusion rate was increased incrementally to reach a minimum duration of 2 hours. This was achieved after 8 initial infusions at 0.25 mg/min (15 mg/hr), without any IARs, change in infusion rate, or infusion interruption. A further decrease of infusion time to 1.5 hours was allowed for patients without new IARs during the last 10 infusions or reported serious adverse events within the last 5 infusions. Each rate increment of 0.083 mg/min (~5 mg/hr) was maintained for 3 consecutive infusions, without any new IARs, change in infusion rate, or infusion interruption, before subsequent rate increases. The number of vials should be determined to be reconstituted based on the individual patient's weight. Each vial of Fabrazyme 35 mg has to be reconstituted with 7.2 ml water for injections and each vial of Fabrazyme 5 mg has to be reconstituted with 1.1 ml water for injections. 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List **Price NI:** £315.08. **IE:** price on application. **Marketing authorisation holder:** Genzyme Europe B.V., Paasheuvelweg 25, 1105 BP Amsterdam, The Netherlands. **Marketing authorisation numbers:** Fabrazyme 5mg powder for concentrate for solution for infusion x 1 vial: EU/1/01/188/004. Fabrazyme 35mg powder for concentrate for solution for infusion x 1 vial: EU/1/01/188/001. **For more information please contact: NI:** Medical Information, Sanofi, 410 Thames Valley Park Drive, Reading, Berkshire, RG6 1PT, UK. uk-medicalinformation@sanofi.com. **IE:** Sanofi, 18 Riverwalk, Citywest Business Campus, Dublin 24 or contact IEmedinfo@sanofi.com **Date of Preparation:** May 2022. **MAT-IE-2200263**

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