Health Technology Briefing
April 2026

Vonaprument for treating geographic atrophy

Company/Developer	Annexon, Inc

☒ New Active Substance ☐ Significant Licence Extension (SLE)

NIHRIO ID: 30994	NICE ID: Not Available	UKPS ID: Not Available

Licensing and Market Availability Plans

Currently in phase III clinical development.

Summary

Vonaprument is currently being developed for the treatment of patients with geographic atrophy (GA) caused by age-related macular degeneration (AMD). GA is an advanced form of "dry" AMD, an eye condition that mainly affects people over the age of 50. It damages the macula, the central part of the retina, a part of the eye responsible for sharp, detailed vision. As GA progresses, areas of retinal tissue are lost, leading to gradual and irreversible loss of central vision. This can make everyday activities such as reading, driving, and recognising faces increasingly difficult. There are currently no recommended treatments for GA in the UK, and the condition has a significant impact on quality of life, highlighting a clear unmet clinical need.

Vonaprument is a medicine given as an injection directly into the eye (intravitreal injection). It is designed to block a protein called C1q, which plays a role in driving inflammation and nerve damage in GA and contributes to the loss of vision. By targeting this process within the eye, vonaprument aims to protect retinal cells and slow vision loss. If licensed, vonaprument could provide an additional treatment option for patients with GA secondary to age-related macular degeneration.

Proposed Indication

This briefing reflects the evidence available at the time of writing and a limited literature search. It is not intended to be a definitive statement on the safety, efficacy or effectiveness of the health technology covered and should not be used for commercial purposes or commissioning without additional information. A version of the briefing was sent to the company for a factual accuracy check. The company was available to comment.

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Treatment of patients with Geographic Atrophy (GA) secondary to age-related macular degeneration (AMD).[1]

Technology

Description

Vonaprument (ANX007) is an antigen-binding fragment (Fab) antibody designed as a first-in-kind therapeutic to selectively inhibit C1q, the initiating molecule of the classical complement pathway and a key driver of neurodegeneration. In advanced dry AMD or GA, C1q binds to photoreceptor synapses, causing aberrant activation of the classical pathway with synapse loss, inflammation and neuronal damage that results in vision loss.[2]

Vonaprument is given intravitreally every month during the 24-month treatment period in the phase III clinical trial ARCHER II (NCT06510816).[1]

Key Innovation

Despite GA being considerably debilitating to patients and carrying a high risk of developing clinical depression, there are currently no recommended treatments for GA in the UK.[3,4] Slowing vision loss in advanced dry AMD is a significant unmet clinical need that has been increasing in the UK over the last decade.[5-7] Vonaprument is a first-in-kind, non-pegylated Fab designed to block C1q locally in the eye with an intravitreal formulation.[2]

If licenced, vonaprument could provide an additional treatment option for patients with GA secondary to AMD.

Regulatory & Development Status

Vonaprument does not currently have Marketing Authorisation in the EU/UK for any indication.

Vonaprument is not in phase II/III clinical development for any other indication.

Vonaprument has the following regulatory designations/awards:

- EMA PRIME designation in the EU received in October 2023[8]
- FDA Fast Track[2]

Patient Group

Disease Area and Clinical Need

The macula is the centre of the retina that is responsible for high acuity vision. AMD is a progressive degenerative disease of the macula typically affecting people over the age of 50 years. There are two types of advanced forms of the disease, commonly called dry and wet AMD. Dry AMD is a slowly deteriorating condition with no treatment for late stage disease at present.[9] GA is the progressive, irreversible, advanced form of dry AMD which is characterised by atrophic lesions in the outer retina and can lead to irreversible central blindness over time. Once lesions progress to the centre of the macula (called the fovea), central vision and visual acuity function are severely impaired. Many people living with GA report difficulty reading, driving, and recognising faces. The most important risk factors for GA are increasing age and family history, while smoking and genetic mutations in the complement system are additional risk factors.[4] AMD affects men and women equally.[9]

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AMD is the most common cause of visual impairment in the older population significantly affecting their quality of life and independence.[6] Twenty-five percent of legal blindness attributed to AMD is accounted for by GA.[10] AMD usually affects people over 50 and is the biggest cause of sight loss in the UK, affecting around 700,000 people. Around one in every 200 people has AMD at 60, however, by the age of 90 it affects one in five people.[9] In England (2024-25) there were 75,496 finished consultant episodes (FCE) and 74,628 admissions for degeneration of macula and posterior pole (ICD-10 code H35.3) which resulted in 73,923 day cases and 937 FCE bed days.[11]

Recommended Treatment Options

There are currently no NICE guidelines for the treatment of patients with Geographic Atrophy (GA) secondary to age-related macular degeneration (AMD).

There is currently no known cure for dry AMD, but several approaches can help manage the condition and potentially slow its progression. These include lifestyle changes like quitting smoking and getting regular exercise, nutritional supplements like the AREDS2 formula, vision aids, and having a diet rich in antioxidants which are contained in foods like leafy greens and certain vegetables.[9]

Self-monitoring is recommended as treatment is required if secondary 'wet'/neovascular AMD develops.[12]

Clinical Trial Information

Trial	**ARCHER II**; NCT06510816; A Phase 3, Multicenter, Randomized, Parallel-Group, Double-Masked, 2-Arm, Sham Controlled Study of the Efficacy, Safety, and Tolerability of ANX007 Administered by Intravitreal Injection in Patients With Geographic Atrophy (GA) Secondary to Age Related Macular Degeneration (AMD) **Phase III**: Active Not Recruiting **Locations:** UK, USA, Canada, 9 EU countries and other countries. **Primary Completion Date:** October 2026
Trial Design	Randomised, Parallel assignment, Quadruple blind.
Population	N=659 (actual); Patients with a diagnosis of GA of the macula secondary to AMD as determined by the Investigator and confirmed by the independent Central Reading Center; aged 50 years and older.
Intervention(s)	Vonaprument intravitreal injections every month during the 24-month treatment period.
Comparator(s)	Sham group intravitreal injections every month during the 24-month treatment period.
Outcome(s)	**Primary outcome measure:** • Proportion of patients experiencing a best corrected visual acuity ≥15-letter loss from baseline through the primary timepoint as assessed by the Early Treatment Diabetic Retinopathy Study chart at any two consecutive visits. The primary timepoint is based on accumulation of the target event rate, but no earlier than Month 12 and no later than Month 18. See trial record for full list of outcomes.

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Results (efficacy)	-
Results (safety)	-

Trial	**ARCHER; NCT04656561;** A Phase 2, Multicenter, Randomized, Parallel-Group, Double-Masked, 4-Arm, Sham-Controlled Study of the Efficacy, Safety, and Tolerability of ANX007 Administered by Intravitreal Injection in Patients With Geographic Atrophy (GA) Secondary to Age-Related Macular Degeneration (AMD) **Phase II**: Completed **Locations:** United States, Australia, and New Zealand **Primary Completion Date:** March 2023
Trial Design	Randomised, Parallel assignment, Quadruple blind
Population	N=270 (actual)
Intervention(s)	Vonaprument, administration via intravitreal injections every month (Group 1) or every other month (Group 2).
Comparator(s)	Sham comparator, administration via intravitreal injections every month (Group 3) or every other month (Group 4).
Outcome(s)	**Primary outcome measure:** • Change in GA lesion area as assessed by fundus autofluorescence (FAF) See trial record for full list of outcomes.
Results (efficacy)	Vonaprument represents the first complement therapeutic candidate to preserve visual acuity, demonstrating a statistically significant reduction of vision loss in both foveal and non-foveal patients at 12 months. Over the 12-month treatment period, patients in the vonaprument monthly treatment group showed a 72% reduction in risk of ≥15-letter vision loss (n=89, p=0.006) and patients in the vonaprument every-other-month treatment group showed a 48% reduction in risk of ≥15-letter vision loss (n=92, p=0.064). Patients in the pooled treatment group showed a 59% reduction in risk of ≥15-letter loss (n=181, p=0.008).[13]
Results (safety)	Treatment with vonaprument was generally well-tolerated in the 181 GA patients treated either monthly or every other month. There was a limited incidence of choroidal neovascularisation conversion across all three groups, with 3.4% in the sham group vs. 4.5% and 4.3% in the vonaprument monthly and every-other-month groups, respectively. Among the patients treated with vonaprument, three serious adverse events (SAEs) of endophthalmitis were observed, which were determined to be related to intravitreal injection procedure and not related to treatment. There were three SAEs of intraocular inflammation, suspected of being treatment-related, which were not associated with retinal vasculitis. One SAE of retinal artery occlusion was reported, suspected of being treatment-related, which was also not associated with retinal vasculitis.[13]

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Estimated Cost

The cost of vonaprument is not yet known.

Relevant Guidance

NICE Guidance

- NICE guideline. Age-related macular degeneration (NG82). January 2018.

NHS England (Policy/Commissioning) Guidance

No guidance identified

Other Guidance

- The Royal College of Ophthalmologists, Commissioning Guidance - Age Related Macular Degeneration Services (2021)[6]

Additional Information

Annexon, Inc did not enter information about this technology onto the UK PharmaScan database; the primary source of information for UK horizon scanning organisations on new medicines in development. As a result, the NIHR Innovation Observatory has had to obtain data from other sources. UK PharmaScan is an essential tool to support effective NHS forward planning; allowing more effective decision making and faster uptake of innovative new medicines for patients who could benefit. We urge pharmaceutical companies to use UK PharmaScan so that we can be assured of up-to-date, accurate and comprehensive information on new medicines.

References

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