Macugen (pegaptanib)
Policy Number: C10418-A

CRITERIA EFFECTIVE DATES:

ORIGINAL EFFECTIVE DATE	LAST REVIEWED DATE	NEXT REVIEW DATE
8/1/2011	5/20/2020	5/20/2021
J CODE	**TYPE OF CRITERIA**	**LAST P&T APPROVAL/VERSION**
J2503-injection, pegaptanib sodium, 0.3mg	RxPA	Q3 2020 20200722C10418-A

PRODUCTS AFFECTED:
Macugen (pegaptanib)

DRUG CLASS:
Vascular endothelial growth factor (VEGF) antagonists

ROUTE OF ADMINISTRATION:
Intravitreal injection

PLACE OF SERVICE:
Buy and Bill
The recommendation is that medications in this policy will be for medical benefit coverage and administered in a place of service that is a non-hospital facility-based location (i.e., home infusion provider, provider's office, free-standing ambulatory infusion center)

AVAILABLE DOSAGE FORMS:
Macugen SOLN 0.3MG 0.3mg/0.09ml (1 PFS)

FDA-APPROVED USES:
Macugen is indicated for the treatment of neovascular (wet) age-related macular degeneration

COMPENDIAL APPROVED OFF-LABELED USES:
treatment of diabetic macular edema, treatment of proliferative diabetic retinopathy

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COVERAGE CRITERIA: INITIAL AUTHORIZATION

DIAGNOSIS:
neovascular (wet) age-related macular degeneration, treatment of diabetic macular edema, treatment of proliferative diabetic retinopathy

REQUIRED MEDICAL INFORMATION:
A. FOR ALL INDICATIONS:

1. Documented diagnosis of neovascular (Wet) age-related macular degeneration, diabetic macular edema or treatment of proliferative diabetic retinopathy
   AND
2. Documentation that member is free of ocular and/or peri-ocular infections
   AND
3. Documentation of trial/failure or contraindication to bevacizumab
   AND
4. Documentation of baseline visual status (visual acuity testing) with notation of eye(s) being

treated

AND

5. Prescriber attests that member will be using Macugen (pegaptanib) as monotherapy, not in combination with other vascular endothelial growth factor (VEGF) inhibitors

DURATION OF APPROVAL:
Initial: 6 months, Continuation: 12 months

QUANTITY:
0.3 mg injection: 1 injection per eye every 42 days

PRESCRIBER REQUIREMENTS:
Prescribed by or in consultation with a board-certified ophthalmologist, ophthalmic surgeon or retinal specialist

AGE RESTRICTIONS:
18 years of age and older

CONTINUATION OF THERAPY:
A. FOR ALL INDICATIONS:

1. Prescriber attests to improvement or stabilization of disease state (ie. increase or stabilization in number of letters during visual acuity testing) AND
2. Documentation of administration records showing dates and eye(s) administered, along with documentation of member compliance with treatment plan
   NOTE: Therapy may be discontinued due to poor adherence upon recommendation of the Molina Medical Director when adherence < 85% has been demonstrated in at least two months during therapy
   AND
3. Documentation of absence of unacceptable toxicity from the drug (i.e. endophthalmitis and retinal detachments; increase in intraocular pressure or arterial thromboembolic events)

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:
All other uses of Macugen (pegaptanib) that are not an FDA-approved indication or not included in this policy are considered experimental/investigational or not a covered benefit of this policy. This subject to change based on research and medical literature, or at the discretion of Molina Healthcare.

OTHER SPECIAL CONSIDERATIONS:
None

BACKGROUND:
Description/Mechanism of Action: Macugen (pegaptanib sodium injection) is a sterile, aqueous solution containing pegaptanib sodium for intravitreous injection. Pegaptanib is a selective vascular endothelial growth factor (VEGF) antagonist. VEGF is a secreted protein that selectively binds and activates its receptors located primarily on the surface of vascular endothelial cells. VEGF induces angiogenesis, and increases vascular permeability and inflammation, all of which are thought to contribute to the progression of the neovascular (wet) form of age-related macular degeneration (AMD), a leading cause of blindness. VEGF has been implicated in blood retinal barrier breakdown and pathological ocular neovascularization. Pegaptanib is an aptamer, a pegylated modified oligonucleotide, which adopts a three-dimensional conformation that enables it to bind to extracellular VEGF. Under in vitro testing conditions, pegaptanib binds to the major pathological

VEGF isoform, extracellular VEGF165, thereby inhibiting VEGF165 binding to its VEGF receptors. The inhibition of VEGF164, the rodent counterpart of human VEGF165, was effective at suppressing pathological neovascularization

APPENDIX:

Administration of intravitreal pegaptanib led to an improvement in visual acuity and a reduction in central retinal thickness in some patients with early stage diabetic macular edema (DME) in a randomized, double- blind, dose-finding, phase-II trial. Patients (n=172) with visual acuity letter scores between 68 and 25 (Snellen equivalent, 20/50 to 20/320) in the study eye, a minimum score of 35 (20/100 or better) in the other eye, and intraocular pressure no greater than 23 mmHg were eligible. Extensive exclusion criteria were employed, leading to enrollment of patients with early stages of DME. Patients were randomized to pegaptanib 0.3 milligrams (mg) (n=44), 1 mg (n=44), or 3 mg (n=42) or sham (placebo) injection (n=42) administered by intravitreal injection every 6 weeks for a minimum of 3 injections (up to 6 injections). Laser photocoagulation was allowed after week 13 if clinically indicated. Study endpoints included visual acuity (VA), central retinal thickness (CRT) on ocular coherence tomography (OCT), and need for laser photocoagulation. Pegaptanib- treated patients received an average of 5 injections, with 49% (83/172) receiving the maximum of 6 injections. Of the patients receiving the active study regimen, the patients allocated to the 0.3-mg dose had greater improvement in VA, greater decrease in retinal thickness, and lesser need for focal/grid laser intervention. The mean changes in VA (in letters) from baseline to 36 weeks were +4.7, +4.7, and +1.1 for pegaptanib 0.3 mg (p=0.04), 1 mg (p=0.05), and 3 mg (p=0.55), respectively, compared to -0.4 in the placebo group. The median VA (in Snellen equivalents) in the 0.3-mg group was better than the placebo group at 36 weeks (20/50 versus 20/63, respectively). The mean change in central retinal thickness from baseline to week 36 was -68 micrometers (range, -118.9 to -9.88) in the pegaptanib 0.3-mg group versus +3.7 micrometers in the placebo group (p=0.02). Twenty-five percent (11/44) of the pegaptanib 0.3-mg group and 48% (20/42) of the placebo group received focal photocoagulation between weeks 12 and 36. Common ocular adverse events that occurred more frequently in the pegaptanib groups included: eye pain (31% versus 17%; p=0.029), vitreous floaters (22% versus 7%; p=0.009), punctuate keratitis (18% versus 17%), cataract (13% versus 10%), eye discharge (11% versus 10%), conjunctival hemorrhage (10% versus 0%), vitreous opacities (9% versus 5%), blurred vision (7% versus 5%), vitreous disorder not otherwise specified (7% versus 0%; p=0.041), and visual disturbances not otherwise specified (7% versus 2%).

REFERENCES:

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