# Everolimus for subependymal giant cell astrocytoma

As a result of a trial, the US Food and Drug Administration (FDA) approved everolimus for patients with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex who are not candidates for curative surgical resection.

Patients ≥ 3 years of age with increasing size of SEGA lesions have had a sustained reduction of SEGA volume on everolimus [1].

## Case series

A retrospective study included TSC patients being treated with oral everolimus for subependymal giant cell astrocytomas (SEGAs) and angiomyolipomas (AMLs). We recorded the changes in facial angiofibroma. Changes in the Angiofibroma Grading Scale (AGS) indicators were recorded according to erythema, average lesion size, lesion density, and percent involvement on the forehead, nose, cheeks, and chin. The scores were recorded before and after the administration of oral everolimus.

Twenty-one patients being treated with oral everolimus were enrolled in this study. The mean age was 20.5 years (range 11-44 years, 4 males, and 17 females). The mean dose of oral everolimus was 3.6 mg/day. Clinically meaningful and statistically significant improvement was observed in erythema ($p = 0.001$), average lesion size ($p < 0.001$), lesion density ($p < 0.001$), and percent involvement ($p < 0.001$). Changes in the AGS findings were statistically significant on the forehead ($p = 0.001$), nose ($p < 0.001$) cheeks ($p < 0.001$), and chin ($p = 0.004$).

Everolimus shows evident improvement and is approved for TSC-associated SEGAs and AMLs. The current study demonstrated the efficacy of oral everolimus in reducing facial angiofibromas, showing the parallel benefits of the treatment protocol for TSC [2].

# Case reports

A 21-year female patient with large bilateral angiomyolipoma (AML) in both kidneys with the longest diameter more than 12.3 cm and subependymal giant cell astrocytoma (SEGA). Treatment with everolimus (EVE) was initiated at a dose of 10.0 mg/day and continued during the following 3 years. Magnetic resonance imaging (MRI) was performed before treatment with everolimus was initiated, and consequently at 12 and 36 months for follow-up of the efficacy of the treatment. After 3 years, the total size of the largest AML decreased by ~24.0% in the longest diameter. A reduction in the total size of SEGA was also observed. The most common adverse effect of treatment was stomatitis grades 3 to 4 and one febrile episodes associated with a skin rash that required a reduced dose of EVE. In conclusion, the everolimus treatment improved even such a large renal AML and the effect persisted during the long-term administration with a small number of adverse effects. A positive effect was observed on the brain tumor as well [3].

A case of a woman with TSC and Multifocal micronodular pneumocyte hyperplasia (MMPH) who received everolimus, for the treatment of a subependymal giant cell astrocytoma (SEGA). After 3 months of therapy, a remarkable decrease in density of all pulmonary MMPH lesions was observed, without any change in size. This shows that everolimus is active on MMPH similarly to its effects on SEGA, renal angiomyolipomas, and pulmonary lymphangioleiomyomatosis in TSC, and suggests that the dysregulated activation of mTOR which characterizes TSC also plays a role in the pathogenesis of MMPH [4].

---

The aim of a study was to evaluate the efficacy of oral everolimus for TSC-associated angiofibromas.

This retrospective study included TSC patients being treated with oral everolimus for subependymal giant cell astrocytomas (SEGAs) and angiomyolipomas (AMLs). We recorded the changes in facial angiofibromas. Changes in the Angiofibroma Grading Scale (AGS) indicators were recorded according to erythema, average lesion size, lesion density, and percent involvement on the forehead, nose, cheeks, and chin. The scores were recorded before and after the administration of oral everolimus.

Twenty-one patients being treated with oral everolimus were enrolled in this study. The mean age was 20.5 years (range 11-44 years, 4 males, and 17 females). The mean dose of oral everolimus was 3.6 mg/day. Clinically meaningful and statistically significant improvement was observed in erythema ($p = 0.001$), average lesion size ($p < 0.001$), lesion density ($p < 0.001$), and percent involvement ($p < 0.001$). Changes in the AGS findings were statistically significant on the forehead ($p = 0.001$), nose ($p < 0.001$) cheeks ($p < 0.001$), and chin ($p = 0.004$).

Everolimus shows evident improvement and is approved for TSC-associated SEGAs and AMLs. The study demonstrated the efficacy of oral everolimus in reducing facial angiofibromas, showing the parallel benefits of the treatment protocol for TSC [5].

# References

1)
Franz DN, Agricola K, Mays M, Tudor C, Care MM, Holland-Bouley K, Berkowitz N, Miao S, Peyrard S, Krueger DA. Everolimus for subependymal giant cell astrocytoma: 5-year final analysis. Ann Neurol. 2015 Dec;78(6):929-38. doi: 10.1002/ana.24523. Epub 2015 Nov 9. PMID: 26381530; PMCID: PMC5063160.

2)
Wei CC, Hsiao YP, Gau SY, Wu YT, Wu CT, Wu MH, Tsai JD. The Efficacy of Everolimus for Facial Angiofibromas in Tuberous Sclerosis Complex Patients Treated for Renal Angiomyolipoma/Subependymal Giant Cell Astrocytoma. Dermatology. 2020 Oct 8:1-6. doi: 10.1159/000510222. Epub ahead of print. PMID: 33032292.

3)
Rambabova Bushljetik I, Lazareska M, Barbov I, Stankov O, Filipce V, Spasovski G. Bilateral Renal Angiomyolipomas and Subependymal Giant Cell Astrocytoma Associated with Tuberous Sclerosis Complex: a Case Report and Review of The Literature. Balkan J Med Genet. 2021 Mar 23;23(2):93-98. doi: 10.2478/bjmg-2020-0017. PMID: 33816078; PMCID: PMC8009567.

4)
Daccord C, Nicolas A, Demicheli R, Chehade H, Hottinger AF, Beigelman C, Lazor R. Effect of

everolimus on multifocal micronodular pneumocyte hyperplasia in tuberous sclerosis complex. Respir Med Case Rep. 2020 Nov 25;31:101310. doi: 10.1016/j.rmcr.2020.101310. PMID: 33312857; PMCID: PMC7720070.

5)

Wei CC, Hsiao YP, Gau SY, Wu YT, Wu CT, Wu MH, Tsai JD. The Efficacy of Everolimus for Facial Angiofibromas in Tuberous Sclerosis Complex Patients Treated for Renal Angiomyolipoma/Subependymal Giant Cell Astrocytoma. Dermatology. 2020 Oct 8:1-6. doi: 10.1159/000510222. Epub ahead of print. PMID: 33032292.

From:
https://neurosurgerywiki.com/wiki/ - **Neurosurgery Wiki**

Permanent link:
**https://neurosurgerywiki.com/wiki/doku.php?id=everolimus_for_subependymal_giant_cell_astrocytoma**

Last update: **2024/06/07 02:52**