

Rapamycin lotion reduces facial tumors caused by tuberous sclerosis, team reports

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Working on a promising treatment for facial tumors tied to tuberous sclerosis complex, from the left, are Adelaide Hebert, M.D.; John Slopis, M.D.; Mary Kay Koenig, M.D.; Joshua Samuels, M.D., M.P.H.; and Hope Northrup, M.D. All are from McGovern Medical School at UTHealth in Houston with the exception of Slopis, who is with The University of Texas MD Anderson Cancer Center.
Credit: PHOTO CREDIT Maricruz Kwon, UTHealth

Addressing a critical issue for people with a genetic disorder called tuberous sclerosis complex (TSC), doctors at The University of Texas Health Science Center at Houston (UTHealth) reported that a skin cream containing rapamycin significantly reduced the disfiguring facial tumors affecting more than 90 percent of people with the condition.

Findings of the multicenter, international study involving 179 people with tuberous [sclerosis](#) complex appear in the journal *JAMA Dermatology*.

"People with tuberous sclerosis complex want to look like everyone else," said Mary Kay Koenig, M.D., the study's lead author, co-director of the Tuberous Sclerosis Center of Excellence and holder of the Endowed Chair of Mitochondrial Medicine at McGovern Medical School at UTHealth. "And, they can with this treatment."

Tuberous sclerosis complex affects about 50,000 people in the United States and is characterized by the uncontrolled growth of non-cancerous tumors throughout the body.

While benign tumors in the kidney, brain and other organs pose the greater health risk, the tumors on the face produce a greater impact on a patient's daily life by making them look different from everyone else, Koenig said.

Koenig's team tested two compositions of facial cream containing [rapamycin](#) and a third with no rapamycin. Patients applied the cream at bedtime for six months.

"Eighty percent of patients getting the study drug experienced a significant improvement compared to 25 percent of those getting the mixture with no rapamycin," she said.

"Angiofibromas on the face can be disfiguring, they can bleed and they can negatively impact quality of life for individuals with TSC," said Kari Luther Rosbeck, president and CEO of the Tuberous Sclerosis Alliance.

"Previous treatments, including laser surgery, have painful after effects. This pivotal study and publication are a huge step toward understanding the effectiveness of topical rapamycin as a treatment option. Further, it is funded by the TSC Research Program at the Department of Defense. We are so proud of this research," Rosbeck said.

Rapamycin is typically given to patients undergoing an organ transplant. When administered by mouth, rapamycin suppresses the immune system to make sure the organ is not rejected.

Rapamycin and tuberous sclerosis complex are linked by a protein called mTOR. When it malfunctions, tuberous sclerosis complex occurs. Rapamycin corrects this malfunction.

Rapamycin was initially used successfully to treat brain tumors caused by [tuberous sclerosis complex](#), so researchers decided to try it on TSC-related facial tumors. Building on a 2010 pilot study on the use of rapamycin to treat TSC-related facial tumors, this study confirmed that a cream containing rapamycin shrinks these tumors.

As the drug's toxicity is a concern when taken by mouth, researchers were careful to check for problems tied to its use on the skin. "It looks like the medication stays on the surface of the skin. We didn't see any appreciable levels in the bloodstreams of those participating in the study," Koenig said.

The Topical Rapamycin to Erase Angiofibromas in TSC—Multicenter Evaluation of Novel Therapy or TREATMENT trial involved 10 test sites including one in Australia. Koenig said additional studies are

needed to gauge the long-term impact of the drug, the optimal dosage and whether the facial cream should be a combined with an oral treatment.

Provided by University of Texas Health Science Center at Houston

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