

Deoxycholic Acid for Dercum Disease: Repurposing a Cosmetic Agent to Treat a Rare Disease

Channi Silence, MS; Shauna M. Rice, MD; Andrew Liteplo, MD; Kathleen McFadden, MD; Nour Al Jalbout, MD; Ahad Alhassan Al Saud, MD; Jennifer Gao; Arianne S. Kourosh, MD, MPH

PRACTICE POINTS

- Dermatologists should consider Dercum disease when encountering a patient with numerous painful lipomas.
- Subcutaneous administration of deoxycholic acid resulted in a notable reduction in pain and size of lipomas by 30% to 68% per radiographic review.
- Deoxycholic acid may provide an alternative therapeutic option for patients who have Dercum disease with substantial tumor burden.

Dercum disease is a rare condition characterized by multiple painful fatty tumors distributed throughout the body. There currently are no US Food and Drug Administration–approved treatments for Dercum disease, and the treatments tried have shown little to no efficacy, leaving many patients with a profoundly negative impact on quality of life. We present a case series of 3 patients who were diagnosed with Dercum disease and were treated with deoxycholic acid (DCA), a therapy approved for adipolysis of submental fat. The patients experienced a reduction in tumor size with radiographic evidence as well as a notable reduction in symptoms.

Cutis. 2023;111:E4-E8.

Dercum disease (or adiposis dolorosa) is a rare condition of unknown etiology characterized by multiple painful lipomas localized throughout the body.^{1,2} It typically presents in adults aged 35 to 50 years and is at

least 5 times more common in women.³ It often is associated with comorbidities such as obesity, fatigue and weakness.¹ There currently are no approved treatments for Dercum disease, only therapies tried with little to no efficacy for symptom management, including analgesics, excision, liposuction,¹ lymphatic drainage,⁴ hypobaric pressure,⁵ and frequency rhythmic electrical modulation systems.⁶ For patients who continually develop widespread lesions, surgical excision is not feasible, which poses a therapeutic challenge. Deoxycholic acid (DCA), a bile acid that is approved to treat submental fat, disrupts the integrity of cell membranes, induces adipocyte lysis, and solubilizes fat when injected subcutaneously.⁷ We used DCA to mitigate pain and reduce lipoma size in patients with Dercum disease, which demonstrated lipoma reduction via ultrasonography in 3 patients.

Case Reports

Three patients presented to clinic with multiple painful subcutaneous nodules throughout several areas of the body and were screened using radiography. Ultrasonography demonstrated numerous lipomas consistent with Dercum disease. The lipomas were measured by ultrasonography to obtain 3-dimensional measurements of each lesion. The most painful lipomas identified by the patients were either treated with 2 mL of DCA (10 mg/mL) or served as a control with no treatment. Patients returned for symptom monitoring and repeat measurements of both treated and untreated lipomas.

Ms. Silence, as well as Drs. Liteplo, McFadden, Al Jalbout, Al Saud, and Kourosh, are from the Massachusetts General Hospital, Boston. Ms. Silence and Dr. Kourosh are from the Department of Dermatology. Dr. Kourosh also is from the Harvard Medical School, Boston. Ms. Rice is from the University of Massachusetts Medical School, Worcester. Ms. Gao is from Harvard College, Boston.

The authors report no conflict of interest.

Correspondence: Channi Silence, MS, 55 Fruit St, BAR 622, Massachusetts General Hospital Department of Dermatology, Boston, MA 02114 (silencechanni@gmail.com).

doi:10.12788/cutis.0752

Two physicians with expertise in ultrasonography measured lesions in a blinded fashion. Photographs were obtained with patient consent.

Patient 1—A 45-year-old woman with a family history of lipomas was diagnosed with Dercum disease that was confirmed via ultrasonography. A painful 1.63×1.64×0.55-cm lipoma was measured on the volar aspect of the left forearm, and a 1.17×1.26×0.39-cm lipoma was measured on the volar aspect of the right wrist. At a follow-up visit 11 months later, 2 mL of DCA was administered to the lipoma on the volar aspect of the left forearm, while the lipoma on the volar aspect of the right wrist was monitored as an untreated control. Following the procedure, the patient reported 1 week of swelling and tenderness of the treated area. Repeat imaging 4 months after administration of DCA revealed reduction of the treated lesion to 0.80×1.48×0.60 cm and growth of the untreated lesion to 1.32×2.17×0.52 cm. The treated lipoma reduced in volume by 34.55%, while the lipoma in the untreated control increased in volume from its original measurement by 111.11% (Table). The patient also reported decreased pain in the treated area at all follow-up visits in the 1 year following the procedure.

Patient 2—A 42-year-old woman with Dercum disease received administration of 2 mL of DCA to a 1.90×1.70×0.90-cm lipoma of the lateral aspect of the left mid thigh and 2 mL of DCA to a 2.40×3.07×0.60-cm lipoma on the volar aspect of the right forearm 2 weeks later. A 1.18×0.91×0.45-cm lipoma of the volar aspect of the left forearm was monitored as an untreated control. The patient reported bruising and discoloration a few weeks following the procedure. At subsequent 1-month and 3-month follow-ups, the patient reported induration in the volar aspect of the right forearm and noticeable reduction in size of the lesion in the lateral aspect of the left mid thigh. At the 6-month follow-up, the patient reported reduction in size of both lesions and improvement of the previously noted side effects. Repeat ultrasonography approximately 6 months after administration of DCA demonstrated reduction of the treated lesion on the lateral aspect of the left mid thigh to 0.92×0.96×0.57 cm and the volar aspect of the right forearm to 1.56×2.18×0.79 cm, with growth of the untreated lesion on the volar aspect of the left forearm to 1.37×1.11×0.39 cm. The treated lipomas reduced in volume by 68.42% and 41.25%, respectively, and the untreated control increased in volume by 22.08% (Table).

Patient 3—A 75-year-old woman with a family history of lipomas was diagnosed with Dercum disease verified by ultrasonography. The patient was administered 2 mL of DCA to a 2.65×3.19×0.71-cm lipoma of the volar aspect of the left forearm. A 1.66×2.02×0.38-cm lipoma of the lateral aspect of the right forearm was monitored as an untreated control. Following the procedure, the patient reported initial swelling that persisted for a few

weeks followed by notable pain relief and a decrease in lipoma size. At 2-month follow-up, the patient reported no pain or other adverse effects, while repeat imaging demonstrated reduction of the treated lesion on the volar aspect of the left forearm to 2.13×2.56×0.75 cm and growth of the untreated lesion on the lateral aspect of the right forearm to 1.95×2.05×0.37 cm. The treated lipoma reduced in volume by 30.29%, and the untreated control increased in volume by 15.05% (Table).

Comment

Deoxycholic acid is a bile acid naturally found in the body that helps to emulsify and solubilize fats in the intestines. When injected subcutaneously, DCA becomes an adipolytic agent that induces inflammation and targets adipose degradation by macrophages, and it has been manufactured to reduce submental fat.⁷ Off-label use of DCA has been explored for nonsurgical body contouring and lipomas with promising results in some cases; however, these prior studies have been limited by the lack of quantitative objective measurements to effectively demonstrate the impact of treatment.^{8,9}












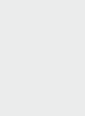
We present 3 patients who requested treatment for numerous painful lipomas. Given the extent of their disease, surgical options were not feasible, and the patients opted to try a nonsurgical alternative. In each case, the painful lipomas that were chosen for treatment were injected with 2 mL of DCA. Injection-associated symptoms included swelling, tenderness, discoloration, and induration, which resolved over a period of months. Patient 1 had a treated lipoma that reduced in volume by approximately 35%, while the control continued to grow and doubled in volume. In patient 2, the treated lesion on the lateral aspect of the mid thigh reduced in volume by almost 70%, and the treated lesion on the volar aspect of the right forearm reduced in volume by more than 40%, while the control grew by more than 20%. In patient 3, the volume of the treated lipoma decreased by 30%, and the control increased by 15%. The follow-up interval was shortest in patient 3—2 months as opposed to 11 months and 6 months for patients 1 and 2, respectively; therefore, more progress may be seen in patient 3 with more time. Interestingly, a change in shape of the lipoma was noted in patient 3 (Figure)—an increase in its depth while the center became anechoic, which is a sign of hollowing in the center due to the saponification of fat and a possible cause for the change from an elliptical to a more spherical or doughnutlike shape. Intralesional administration of DCA may offer patients with extensive lipomas, such as those seen in patients with Dercum disease, an alternative, less-invasive option to assist with pain and tumor burden when excision is not feasible. Although treatments with DCA can be associated with side effects, including pain, swelling, bruising, erythema, induration, and numbness, all 3 of our patients had ultimate mitigation of pain and reduction in lipoma size within months of the injection. Additional studies should be

Lipoma Dimensions and Volumes of 3 Patients With Dercum Disease

Lesion number/site	Lesion dimensions at presentation	3D rendering of lesion at presentation	Lesion dimensions after DCA treatment	3D rendering of lesion at follow-up ^a
Patient 1				
Lesion 1/volar aspect of left forearm (treated)	T	1.63 cm	0.80 cm	
	S	1.64 cm	1.48 cm	
	D	0.55 cm	0.60 cm	
	V	1.65 cm ³	1.08 cm ³	
	Change in V		-34.55%	
Lesion 2/volar aspect of right wrist (untreated control)	T	1.17 cm	1.32 cm	
	S	1.26 cm	2.17 cm	
	D	0.39 cm	0.52 cm	
	V	0.90 cm ³	1.90 cm ³	
	Change in V		+111.11%	
Patient 2				
Lesion 1/lateral aspect of left mid thigh (treated)	T	1.90 cm	0.92 cm	
	S	1.70 cm	0.96 cm	
	D	0.90 cm	0.57 cm	
	V	2.47 cm ³	0.78 cm ³	
	Change in V		-68.42%	
Lesion 2/volar aspect of right forearm (treated)	T	2.40 cm	1.56 cm	
	S	3.07 cm	2.18 cm	
	D	0.60 cm	0.79 cm	
	V	4.17 cm ³	2.45 cm ³	
	Change in V		-41.25%	

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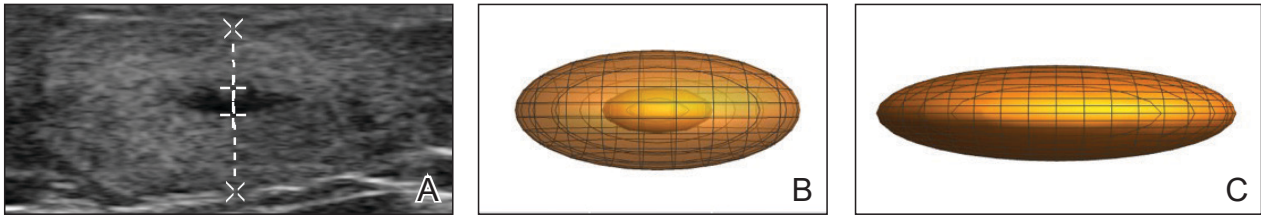
TABLE. (continued)

Lesion number/site	Lesion dimensions at presentation	3D rendering of lesion at presentation	Lesion dimensions after DCA treatment	3D rendering of lesion at follow-up ^a
Patient 2 (continued)				
Lesion 3/volar aspect of left forearm (untreated control)	T	1.18 cm	1.37 cm	
	S	0.91 cm	1.11 cm	
	D	0.45 cm	0.39 cm	
	V	0.77 cm ³	0.94 cm ³	
	Change in V		+22.08%	
Patient 3				
Lesion 1/volar aspect of left forearm (treated)	T	2.65 cm	2.13 cm	
	S	3.19 cm	2.56 cm	
	D	0.71 cm	0.75 cm	
	V	4.82 cm ³	3.36 cm ³	
	Change in V		-30.29%	
Lesion 2/lateral aspect of right forearm (untreated control)	T	1.66 cm	1.95 cm	
	S	2.02 cm	2.05 cm	
	D	0.38 cm	0.37 cm	
	V	1.86 cm ³	2.14 cm ³	
	Change in V		+15.05%	

Abbreviations: D, depth; DCA, deoxycholeic acid; S, sagittal; T, transverse; V, volume.

^aFollow-up was conducted at 11 months for patient 1, 6 months for patient 2, and 2 months for patient 3. In treated lesions, follow-up was after treatment with DCA. No treatment was applied to the control lesions.

3D renderings courtesy of Jennifer Gao (Boston, Massachusetts).



A, Ultrasonography of a lipoma on the volar aspect of the left forearm at 2-month follow-up that was treated with 2 mL of deoxycholic acid (DCA) in a patient with Dercum disease (patient 3). It has an anechoic center at the site of saponification of fat after the injection of DCA. B, A 3D model of the treated lipoma showed a more spherical shape with a hollow center. C, A 3D model of the treated lipoma after being compressed. 3D renderings courtesy of Jennifer Gao (Boston, Massachusetts).

explored to determine the optimal dose and frequency of administration of DCA that could benefit patients with Dercum disease.

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