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Biotech upstart joins Big Pharma cast on high-profile 'breakthrough' stage

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Up until now, the FDA has reserved its new "breakthrough" designation for a Who's Who in drug development, with high-profile companies like J&J, Merck and Novartis touting their scores at the agency. But now the FDA has delivered the coveted designation to Durham, NC-based ScioDerm, a little-known start-up that just landed its Series A of $16 million. And its success is blazing a path others can follow.

ScioDerm won breakthrough status for SD-101, a topical formulation used to heal the skin of patients with rare cases of Epidermolysis Bullosa, a condition where skin is extraordinarily fragile and thin, causing it to tear easily. In a small Phase II proof-of-concept study with 8 patients, 7 experienced a complete closure of targeted lesions within a month. The company says the drug also significantly reduced lesions after three months and was well-tolerated by the kids.

Robert Ryan is the CEO of the biotech, which has a virtual staff of 6. He's been a regulatory exec for Quintiles and PPD, among others, and he says the key to becoming the first little biotech to achieve this regulatory path has been going out and gathering human proof-of-concept data early on in a field in which developers often initially lean heavily on animal data.

"The key part is believing in your product and doing the appropriate testing to see if your product works," Ryan tells *FierceBiotech*. Breakthrough status requires some compelling PoC data. And the big advantage, he adds, is that the FDA has now committed to putting together a cross-disciplinary team to work directly with him on the clinical development process. Getting a meeting with senior-level regulators and gathering direct feedback on development should not be an issue, he adds, and that's a very big plus for a small company in a hurry.

Just days ago, Morgenthaler Ventures and Technology Partners announced they had put together the Series A round for the company, which is aiming to get another, possibly pivotal, clinical study of SD-101 ramped up before the end of the year. And Ryan says that the company now has the financing to possibly make it all the way to registration, which could come in two years if all goes according to plan. That's an ambitious timeline, but with good contacts at the FDA and the right data from a pivotal study, ScioDerm executives believe they have a good shot at success.

- read the release on the FDA designation
- here's the release on the Series A