

Pediatric Research and Equity Act Waivers

- Laviv

BLA #: 125348

Supplement Type: N/A Supplement Number: N/A

Product name and active ingredient/dosage form:

- **Product name: azficel-T**
- **Active ingredient: a suspension of autologous cultured fibroblasts expanded from a patient's skin biopsy**
- **Dosage: 0.1 mL/linear cm (1.0-2.0 x 10⁷ cells/mL), up to 2 mL/Tx. by intradermal injections. In the two pivotal clinical trials, a course of treatment consisted of three treatment sessions scheduled five weeks apart.**

Sponsor: **Fibrocel Technology**

Indications(s): **Treatment of moderate to severe nasolabial fold wrinkles in adults**
(NOTE: If the drug is approved for or Sponsor is seeking approval for more than one indication, address the following for each indication.)

1. Pediatric age group(s) to be waived.

Up to age 18

2. Reason(s) for waiving pediatric assessment requirements (choose all that apply **and provide justification**):
 - a. Studies are impossible or highly impractical (e.g. the number of pediatric patients is so small or is geographically dispersed). If applicable, chose from adult-related conditions in Attachment I
 - b. The product would be ineffective or unsafe in one or more of the pediatric group(s) for which a waiver is being requested. Note: If this is the reason the studies are being waived, this information **MUST** be included in the pediatric use section of labeling. Please provide the draft language you intend to include in the label. Suggested language includes, "FDA has not required pediatric studies in ages ____ to ____ because (state the safety or effectiveness reason)."
 - c. The product fails to represent a meaningful therapeutic benefit over existing therapies for pediatric patients **and** is unlikely to be used in a substantial number of all pediatric age groups or the pediatric age group(s) for which a waiver is being requested.

The product is a human autologous cell therapy which has very limited applicability to pediatric patients for the improvement of nasolabial fold wrinkles because this condition occurs only in the adult population. Development of facial wrinkles is a typical component of the intrinsic aging process of the skin, and is often exacerbated by accumulated exposure to ultraviolet light radiation. Although it has been estimated that 80% of photodamage has already occurred before a person turns 18, literature reports indicate that photoaging symptoms take at least a decade to emerge.

Of skin aging and wrinkle conditions that occur in children (progeroid syndromes), this product is not applicable as it is hypothesized that progeroid syndromes may be caused by alterations in DNA repair and stability, mutations in genes encoding lamins A/C (an intermediate filament protein), or altered mitochondrial function. Although therapeutic approaches have so far been inconclusive, research has been mainly focused on trying to restore these defective subcellular mechanisms. For this reason, this product does not represent a meaningful therapeutic benefit for progeroid syndrome affected children.

The American Society of Plastic Surgeons reported in 2007 that patients 13-19 years of age accounted for fewer than 0.5% of cosmetic procedures used for the reduction and aesthetic improvement of facial skin wrinkles. It is unlikely that this product would be used in a substantial number of pediatric patients, and enrollment of pediatric studies would be difficult and impracticable.

- d. Reasonable attempts to produce a pediatric formulation for one or more of the pediatric age group(s) for which the waiver is being requested have failed. (Provide documentation from Sponsor) Note: Sponsor must provide data to support this claim for review by the Division, and this report submitted by the Sponsor will be publicly posted.

Attachment I

Adult-Related Conditions that do not occur in pediatrics and qualify for a waiver

These conditions qualify for waiver because studies would be impossible or highly impractical

Age-related macular degeneration
Alzheimer's disease
Amyotrophic lateral sclerosis
Atherosclerotic cardiovascular disease
Benign prostatic hypertrophy
Chronic Obstructive Pulmonary Disease
Erectile Dysfunction
Infertility

Cancer:
Basal cell
Bladder
Breast
Cervical
Colorectal
Endometrial
Gastric

Menopausal and perimenopausal disorders	Hairy cell
leukemia	
Organic amnesic syndrome	Lung (small & non-small cell)
(not caused by alcohol or other psychoactive substances)	Multiple myeloma
Osteoarthritis	Oropharynx (squamous cell)
Parkinson's disease	Ovarian (non-germ cell)
Postmenopausal Osteoporosis	Pancreatic
Vascular dementia/ Vascular cognitive disorder/impairment	Prostate
	Renal cell
	Uterine

Page Last Updated: 01/29/2015

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