

NDC	DRUG PROD DESC	INTRODUCED TO MARKET DATE	WAC AT INTRODUCTION
73554-2111-01	SKYSONA is a cell suspension for intravenous infusion; a single dose of SKYSONA contains a minimum of 5.0×10^6 CD34+ cells/kg of body weight, suspended in a solution containing 5% dimethyl sulfoxide (DMSO)	12/1/2022	3,000,000.00

MARKETING PRICING PLAN	MARKETING PRICING NONPUBLIC	ESTIMATED PATIENTS	BREAKTHROUGH THERAPY INDICATOR
SKYSONA is an autologous hematopoietic stem cell-based gene therapy indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD). CALD is a rare, progressive, neurodegenerative disease that primarily affects young boys and causes irreversible, devastating neurologic decline, including major functional disabilities such as loss of communication, cortical blindness, requirement for tube feeding, total incontinence, wheelchair dependence, or complete loss of voluntary movement. Nearly half of patients who do not receive treatment die within five years of symptom onset. bluebird took this profound impact into consideration when pricing the therapy—weighing the therapy’s robust and sustained clinical benefit, quality of life improvements for patients and their families, cost savings to the system, and the overall impact on society. We are focused on timely access to this therapy for this patient population.	N	40	Y

PRIORITY REVIEW INDICATOR	ACQUISITION DATE	ACQUISITION PRICE	ACQUISITION PRICE NONPUBLIC	ACQUISITION PRICE COMMENT
Y				

GENERAL COMMENTS