Kebilidi™ (eladocagene exuparvovec-tneq)

Program Enrollment Form for Referring Physicians

PTC Cares™

TO BE COMPLETED BY REFERRING HEALTHCARE PROVIDER

Please complete all required fields to initiate enrollment for your patient into PTC *Cares*™.
Return via Fax to 1-877-204-2180. Please call PTC Cares at 1-844-478-2227 with questions.

PATIENT INFORMATION

Patient Name (First, MI, Last)	Date of Birth / /	☐ Male ☐ Female	
Guardian/Caregiver's Name	Relationship		
Address	City	State	ZIP
Home Phone ☐ OK to leave message	Mobile	☐ OK to leave message	
Preferred Contact Number ☐ Home ☐ Mobile	Best time to reach me ☐ Morning ☐ Afternoon ☐ Evening		
Email Address	Primary Language ☐ English ☐ Spanish ☐ Other		

CLINICAL INFORMATION

Confirmed AADC Deficiency Diagnosis ☐ Yes ☐ No
Confirmatory Testing Completed (Select all that apply): Genetic Testing ☐ Neurotransmitter Testing ☐ AADC Enzyme Testing ☐
Additional Information (include diagnostic test results as attachments to this form if possible)

REFERRING PHYSICIAN INFORMATION

Physician Name (First, Last)				
Affiliated Hospital/Organization				
Address		City	State	ZIP
Phone	Fax	NPI#	DEA#	Tax ID#
Office Contact		Phone	Best time to contact ☐ Morning ☐Afternoon	
Office Contact Email				

Physician Authorization:

By signing the Enrollment Form, I authorize the release of clinical and/or other patient information to agents of PTC Therapeutics, Inc., and service providers to use and disclose as necessary.

Physician Authorization Signature (stamps not acceptable)	Date

Please see **www.KEBILIDI.com** for full Prescribing Information

Contact us: 1-844-4PTCCARES (1-844-478-2227)
Fax completed form to: 1-877-204-2180
Learn more at: www.KEBILIDI.com

Kebilidi™ (eladocagene exuparvovec-tneq)

PTC Cares™

TO BE COMPLETED BY PATIENT/CAREGIVER

Patient Authorization to Share Personal Health Information

PATIENT INFORMATION

Patient Name (First, MI, Last)		Date of Birth / /	☐ Male ☐ Female ☐
Guardian/Caregiver's Name		Relationship	
Address		City	State ZIP
Home Phone	☐ OK to leave message	Mobile	☐ OK to leave message
Preferred Contact Number ☐ Home ☐ Mobile		Best time to reach me ☐ Morning ☐ Afternoon ☐ Evening	
Email Address		Primary Language ☐ English ☐ Spanish ☐ Other	

INSURANCE INFORMATION

	Primary Insurance	Secondary Insurance
Insurance Name		
Policy Number		
Group Number		
Phone Number		
Policyholder Name		
Rx Member ID		
Rx BIN or PCN (if applicable)		
Rx Group ID		

☐ Patient has no insurance
☐ Send copy front/back of prescription, medical, secondary insurance cards.

Patient Authorization and Program Participation:

I have read and agree to the following Patient Authorization to share my health information and participate in the PTC Cares™ program. I authorize my healthcare providers and health plans to disclose personal and health information related to my use or potential use of KEBILIDI™ (eladocagene exuparvovec) to PTC Therapeutics, Inc. and its agents and contractors including, but not limited to, PTC's specialty pharmacy partners and authorize PTC Therapeutics, its agents, and my pharmacies or designated treatment center to use such information to: 1) determine benefit eligibility; 2) communicate with my healthcare providers and health plans about benefit, coverage and medical care; 3) provide me with support services for KEBILIDI™ (eladocagene exuparvovec); 4) contact me and leave messages about KEBILIDI™ (eladocagene exuparvovec); 5) provide me with information or materials related to KEBILIDI™ (eladocagene exuparvovec) or my relevant medical conditions; 6) contact me about the PTC Cares™ program, which may include patient services such as education, training, nurse and pharmacy support; and 7) I understand that my pharmacy may receive remuneration in exchange for sharing and using my information pursuant to this authorization. PTC Therapeutics will maintain the confidentiality of my personal and health information in accordance with its privacy policy and will use this information only for the purposes described above or as permitted by law. However, I understand that once information about me is released based on this authorization, federal and state privacy laws may not prevent PTC Therapeutics from further disclosing my information. I understand that PTC Therapeutics has agreed to only use or disclose information it receives for the purposes described in this authorization or as required by law. I further understand that I may refuse to sign this authorization and that my refusal to sign this authorization will have no impact on my eligibility to receive health plan benefits or treatments from my healthcare providers, but I will not have access to support services from the PTC Cares™ program. I understand that I have the right to revoke this authorization at any time in the future, by submitting a written notice to PTC Therapeutics via fax to 1-908-222-7231 or by mail to PTC Therapeutics, Inc. **Attention**: Compliance Officer, PTC Therapeutics, 500 Warren Corporate Center Drive, Warren, NJ 07059. I understand that after I have revoked my authorization, PTC Therapeutics will no longer disclose my information, except to the extent that action has been taken in reliance on this authorization or administration of the program for record keeping purposes of my participation. I am entitled to a copy of this authorization, which expires 10 years from the date it is signed by me (unless earlier termination is required by applicable state law). For information about how PTC Therapeutics handles your information, please see our privacy statement at PTCBio.com for Privacy Rights and Choices specific to California residents which are available here. The personal, insurance and information I have provided on this form is complete and accurate to the best of my knowledge. I will update my information promptly if any of the information reflected on this form changes by contacting PTC Cares™ at 1-844-478-2227.

Patient/Guardian Signature	
Patient/Guardian Printed Name	
Relationship	Date

Please see **www.KEBILIDI.com** for full Prescribing Information

Contact us: 1-844-4PTCCARES (1-844-478-2227)
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Important Safety Information and indication for KEBILIDI™ (eladocagene exuparvovec-tneq)

Indication
KEBILIDI is an adeno-associated virus vector-based gene therapy indicated for the treatment of adult and pediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency.

This indication is approved under accelerated approval based on change from baseline in gross motor milestone achievement at 48 weeks post-treatment. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory clinical trial.

IMPORTANT SAFETY INFORMATION

Contraindications
KEBILIDI is contraindicated in patients who have not achieved skull maturity assessed by neuroimaging. Skull maturity is needed for stereotactic neurosurgical administration of KEBILIDI.

Warnings and Precautions
Procedure-Related Monitoring: Procedural complications have been reported after neurosurgery required for KEBILIDI administration. These events included respiratory and cardiac arrest which occurred within 24 hours of the neurosurgical procedure and during post-surgical care. KEBILIDI administration has the potential risk for additional procedure related adverse events including cerebrospinal fluid (CSF) leak, intracranial bleeding, neuroinflammation, acute infarction, and infection. Patients should be monitored for procedure related adverse events with KEBILIDI administration.

Dyskinesia: Dyskinesia was reported after administration of KEBILIDI. All events were reported within 3 months of administration and 2 events required hospitalization. Monitor patients for signs and symptoms of dyskinesia after KEBILIDI treatment which may include involuntary movements of face, arm, leg, or entire body. These may present as fidgeting, writhing, wriggling, head bobbing or body swaying. The use of dopamine antagonists may be considered to control dyskinesia symptoms.

Adverse Reactions
The most common adverse reactions (≥15%) in patients treated with KEBILIDI were dyskinesia (77%), pyrexia (38%), hypotension (31%), anemia (31%), salivary hypersecretion (23%), hypokalemia (23%), hypophosphatemia (23%), insomnia (23%), hypomagnesemia (15%), and procedural complications, including respiratory and cardiac arrest (15%).

Use in Specific Populations

- **Pediatric Use:** The safety and efficacy of KEBILIDI have not been studied in pediatric patients younger than 16 months.
- **Geriatric Use:** Clinical studies of KEBILIDI did not include patients 65 years of age and older.

You may also report adverse events directly to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. For product complaints or to report an adverse event, please call 1-866-562-4620 or email at usmedinfo@ptcbio.com.

Please see **www.KEBILIDI.com** for the full Prescribing Information for additional information.

US-KEB-0203 | 11/24