Rocket Pharmaceuticals, Inc
CIRM Access and Affordability Plan for KRESLADI ™ (marnetegragene autotemcel)

1. Therapy description and context

Rocket Pharmaceuticals, Inc. ("Company") is the Commercializing Entity for KRESLADI™ ("KRESLADI" or the "Therapy"), an *ex vivo,* autologous gene therapy indicated as a one-time treatment for pediatric patients with severe LAD-I and expected to be covered under a medical insurance benefit rather than the pharmacy benefit. The patient population is pediatric, such that a parent or legal guardian will generally be the primary insurance policy holder and the primary contact with payers, government programs, and the Company's patient support services.

This Access and Affordability Plan ("Access Plan") describes how the Company will provide access pathways and financial assistance for eligible pediatric patients legally residing in California who have no other means to obtain the Therapy, consistent with CIRM requirements.

2. Purpose and scope

The purpose of this Access Plan is to ensure that eligible, uninsured Californians with no other means to obtain KRESLADI have a meaningful pathway to access the Therapy through clearly defined eligibility, financial assistance, and access support mechanisms, consistent with CIRM requirements and prevailing patient assistance program standards for gene therapy products.

For purposes of this Plan, "no other means" refers to eligible, severe LAD-I pediatric patients who, through their parent or legal guardian:

- Lack coverage or the ability to obtain coverage for the Therapy under any private or government insurance program; and
- Have a household income below 300% of the Federal Poverty Level (FPL), based on household size.

This Plan is limited to access and affordability provisions for eligible California legal residents under CIRM's framework and does not describe the Company's broader U.S. or ex-U.S. market access, pricing, or reimbursement strategy.

In designing this Access Plan, the Company has considered relevant factors identified in CIRM's guidance, including market size, company size, and affordability [redacted]

3. Eligibility criteria and free-drug Patient Assistance Program (PAP)

3.1 Enrollment

- The severe LAD-I pediatric patient, through their parent or legal guardian, must enroll in the Company's patient support program by completing required enrollment and authorization forms and providing requested documentation to allow eligibility determination and coordination of services.

3.2 Confidentiality

- As a condition of participation in the Patient Assistance Program, the parent or legal guardian may be required to sign a confidentiality acknowledgment relating solely to the Company's non-public program materials, operational processes, and proprietary business information disclosed in connection with program administration. Any such acknowledgment will not restrict the patient, parent, or legal guardian from discussing the patient's medical condition or treatment experience, seeking medical care, communicating with healthcare providers, payers, or government agencies, reporting adverse events or product complaints, or making any disclosure protected or required by law.

3.3 Residency and legal status

- The patient (through the parent/legal guardian) is a legal resident of California, as documented by acceptable proof of residency [redacted]
- The patient is a U.S. citizen, lawful permanent resident defined as 1 year in the state of California, or otherwise lawfully present in the United States, as documented by acceptable proof of legal status [redacted]

3.4 Insurance status ("no other means")

The PAP is available only to patients who otherwise meet all clinical, residency, legal status, and income criteria and who have no medical insurance coverage and no ability to

obtain such coverage for the Therapy through any private or government insurance program at the time of enrollment in the PAP.

[redacted]

[redacted]

3.5 Financial eligibility

The patient's household income, as determined based on household size, is verified to be below 300% of the Federal Poverty Level, using the most recent available income documentation [redacted]

- The Company may require periodic recertification of income eligibility consistent with standard patient assistance program practices on a case-by-case basis.

3.6 Clinical eligibility

- The treating physician has attested that the patient's diagnosis and intended use are consistent with the FDA-approved indication for KRESLADI.
- The treatment order is issued by a qualified treating physician at a qualified treatment center participating in the KRESLADI program.

3.7 Insurance navigation as part of eligibility

As part of the patient services and eligibility process, the Company will make reasonable efforts to assist uninsured patients and their families or legal guardians in evaluating and pursuing available third-party coverage options, including government programs such as Medi-Cal and commercial insurance, where appropriate.

[redacted]

[redacted]

3.8 Documentation

The Company will require documentation sufficient to support eligibility determinations.

[redacted]

The Company may update or refine documentation requirements over time to reflect operational experience, changes in applicable requirements, and best practices for patient assistance programs.

4. Patient and provider access support & navigation

The Company operates a patient services program to support eligible families and qualified treating centers with access-related questions.

[redacted]

[redacted]

[redacted]

[redacted]

5. Financial assistance (Patient Assistance Program Scope)

For eligible patients who meet the Access Plan's residency, legal status, income, insurance, and clinical criteria per their healthcare provider, the Company will provide KRESLADI at no cost to the patient through a free-therapy program. [redacted]

The Company will not provide coverage for administration, facility fees, or other non-drug medical services under this Access Plan. [redacted]

6. Equity, language, and cultural access

The Company will design and operate the Access Plan to promote equitable access for eligible, uninsured legal residents of the state of California. To this end, the Company will where feasible and consistent with available resources:

- Work with qualified treatment centers to identify and, where possible, address barriers faced by patients from rural or underserved communities in reaching participating centers.

These efforts will be proportionate to the Company's size and resources and aligned with prevailing patient assistance program norms for *ex vivo,* autologous gene therapies.

7. Post-treatment and follow-up support

KRESLADI is a one-time *ex vivo* autologous gene therapy that does not involve chronic maintenance dosing. Consistent with the Therapy's labeling and applicable regulatory or risk-management requirements, the Access Plan will include limited post-treatment support, and will not provide ongoing case management or long-term non-medical support beyond what is required to support access.

8. Governance, monitoring, and program adjustment

The Company will maintain internal governance for the Access Plan, including:

- [redacted]
- [redacted]
- Adjustments to program processes, communications, and support offerings over time, as appropriate, to ensure that the Access Plan remains operationally feasible, aligned with prevailing industry standards for *ex vivo*, autologous gene therapy products, and consistent with the Company's resources and the "no other means" objective.

The Company may revise this Access Plan over time to reflect operational experience, changes in applicable requirements, and available resources, while maintaining alignment with CIRM's applicable access requirements.