**Louisiana Medicaid**
**Cystic Fibrosis, Oral**

The *Louisiana Uniform Prescription Drug Prior Authorization Form* should be utilized to request clinical authorization for ivacaftor (Kalydeco®), lumacaftor/ivacaftor (Orkambi®) and tezacaftor/ivacaftor (Symdeko®).

Additional Point-of-Sale edits may apply.

*~~NOTE: These~~NOTE: These agents are mutation-specific targeted therapy that ~~is~~are indicated to treat only the cystic fibrosis transmembrane conductance regulator (CFTR) mutation(s) ~~that is~~ listed ~~here~~in the prescribing information. Identification of the indicated genotype is required in order to receive treatment with one of th~~i~~ese agents. If the recipient's genotype is unknown, an FDA-cleared CF mutation test should be used to confirm the presence of a CFTR mutation, followed by verification with bi-directional sequencing when recommended by the mutation test instructions.*

---

**Ivacaftor (Kalydeco®)**

**Approval Criteria**

- The recipient is 4 months old or older on the date of the request with a documented diagnosis of cystic fibrosis; **AND**
- 
- The following is **true** and is ~~**noted**~~ **stated** **on the request**:
  The recipient has ~~-one of the following~~ mutation~~s~~ in the CFTR gene that is responsive to ivacaftor based on clinical and/or *in vitro* assay data~~and the mutation is listed on the request~~; **AND**
- 

| ~~List of CFTR Gene Mutations that Produce CFTR Protein and are Responsive to KALYDECO®~~ | | | | | |
|---|---|---|---|---|---|
| ~~E56K~~ | ~~G178R~~ | ~~S549R~~ | ~~S977F~~ | ~~F1074L~~ | ~~2789+5G→A~~ |
| ~~P67L~~ | ~~E193K~~ | ~~G551D~~ | ~~F1052V~~ | ~~D1152H~~ | ~~3272-26A→G~~ |
| ~~R74W~~ | ~~L206W~~ | ~~G551S~~ | ~~K1060T~~ | ~~G1244E~~ | ~~3849+10kbC→T~~ |
| ~~D110E~~ | ~~R347H~~ | ~~D579G~~ | ~~A1067T~~ | ~~S1251N~~ | |
| ~~D110H~~ | ~~R352Q~~ | ~~711+3A→G~~ | ~~G1069R~~ | ~~S1255P~~ | |
| ~~R117C~~ | ~~A455E~~ | ~~E831X~~ | ~~R1070Q~~ | ~~D1270N~~ | |
| ~~R117H~~ | ~~S549N~~ | ~~S945L~~ | ~~R1070W~~ | ~~G1349D~~ | |

  - ~~The recipient is not homozygous for the F508del mutation in the CFTR gene; **AND**~~
- If the request is for a non-preferred agent - **ONE** of the following is required:
  - The recipient has had a *treatment failure* with at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has had an *intolerable side effect* to at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has *documented contraindication(s)* to the preferred drugs that are appropriate to use for the condition being treated; **OR**
  - There is *no preferred product that is appropriate* to use for the condition being treated; **OR**

- The prescriber states that the recipient is currently using the requested medication and has had a positive clinical response to treatment; **AND**

- By submitting the authorization request, the prescriber attests to the following:
  - ~~The mutation in the CFTR gene is responsive to Kaylydeco® based on clinical and/or vitro assay data; **AND**~~
  - ~~If the recipient's genotype is unknown, an FDA-cleared cystic fibrosis mutation test will be used to detect the presence of a CFTR mutation, followed by verification with bi-directional sequencing when recommended by the mutation test instructions; **AND**~~
  - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning, Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
  - All laboratory testing and clinical monitoring recommended in the prescribing information have been completed as of the date of the request and will be repeated as recommended; **AND**
  - The recipient has no concomitant drug therapies or disease states that limit the use of Kalydeco® and will not be receiving Kalydeco® in combination with any medication that is contraindicated or not recommended per FDA labeling.

**Reauthorization Criteria**

- The recipient continues to meet all initial approval criteria; **AND**
- The prescriber **states on the request** that there is evidence of a positive response to treatment.

**Duration of ~~i~~Initial and r~~R~~eauthorization a~~A~~pproval: 12 months**

---

**Lumacaftor/Ivacaftor (Orkambi®)**

**Approval Criteria**

- Recipient is 2 years of age or older on the date of the request with a documented diagnosis of cystic fibrosis; **AND**
- The following is **true** and is ~~**noted**~~ **stated on the request**: The recipient is homozygous for the *F508del* mutation in the ~~cystic fibrosis transmembrane conductance regulator (~~CFTR~~)~~ gene; **AND**
- If the request is for a non-preferred agent - **ONE** of the following is required:
  - The recipient has had a *treatment failure* with at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has had an *intolerable side effect* to at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has *documented contraindication(s)* to the preferred drugs that are appropriate to use for the condition being treated; **OR**
  - There is *no preferred product that is appropriate* to use for the condition being treated; **OR**

- The prescriber states that the recipient is currently using the requested medication and has had a positive clinical response to treatment; **AND**

- By submitting the authorization request, the prescriber attests to the following:
  - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning, Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
  - All laboratory testing and clinical monitoring recommended in the prescribing information have been completed as of the date of the request and will be repeated as recommended; **AND**
  - The recipient has no concomitant drug therapies or disease states that limit the use of Orkambi® and will not be receiving Orkambi® in combination with any medication that is contraindicated or not recommended per FDA labeling.

**Reauthorization Criteria**

- The recipient continues to meet all initial approval criteria; **AND**
- The prescriber **states on the request** that there is evidence of a positive response to treatment.

**Duration of ~~i~~Initial and ~~r~~Reauthorization ~~a~~Approval: 12 months**

---

**Tezacaftor/Ivacaftor (Symdeko®)**

**Approval Criteria**

- ~~Recipient~~ The recipient is 6 years of age or older; **AND**
- ~~Recipient~~ The recipient has a diagnosis of cystic fibrosis (CF) and **ONE** of the following is ~~**documented**~~ **stated on the request**:
  - The recipient is homozygous for the *F508del* mutation; **OR**
  - The recipient has at least one mutation in the ~~cystic fibrosis transmembrane conductance regulator (~~CFTR~~)~~ gene that is responsive to tezacaftor/ivacaftor based on *in vitro* data and/or clinical evidence~~; AND.~~

| ~~List of CFTR Gene Mutations Responsive to Tezacaftor/Ivacaftor~~ | | | | | | | |
|---|---|---|---|---|---|---|---|
| ~~A1067T~~ | ~~A455E~~ | ~~D110E~~ | ~~D110H~~ | ~~D1152H~~ | ~~D1270N~~ | ~~D579G~~ | ~~E193K~~ |
| ~~E56K~~ | ~~E831X~~ | ~~F1052V~~ | ~~F1074L~~ | ~~K1060T~~ | ~~L206W~~ | ~~P67L~~ | ~~R1070W~~ |
| ~~R117C~~ | ~~R347H~~ | ~~R352Q~~ | ~~R74W~~ | ~~S945L~~ | ~~S977F~~ | ~~F508del (2 copies)~~ | |
| ~~2789+5G→A~~ | | ~~3272-26A→G~~ | | ~~3849+10kbC→T~~ | | ~~711+3A→G~~ | |

- If the request is for a non-preferred agent - **ONE** of the following is required:
  - The recipient has had a *treatment failure* with at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has had an *intolerable side effect* to at least one preferred drug that is appropriate to use for the condition being treated; **OR**
  - The recipient has *documented contraindication(s)* to the preferred drugs that are appropriate to use for the condition being treated; **OR**

- There is *no preferred product that is appropriate* to use for the condition being treated; **OR**
- The prescriber states that the recipient is currently using the requested medication and has had a positive clinical response to treatment; **AND**

- By submitting the authorization request, the prescriber attests to the following:
  - ~~The mutation in the CFTR gene is responsive to Symdekoa® based on clinical and/or vitro assay data; **AND**~~
  - ~~If the recipient's genotype is unknown, an FDA-cleared cystic fibrosis mutation test will be used to detect the presence of a CFTR mutation, followed by verification with bi-directional sequencing when recommended by the mutation test instructions; **AND**~~
  - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning, Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
  - All laboratory testing and clinical monitoring recommended in the prescribing information have been completed as of the date of the request and will be repeated as recommended; **AND**
  - The recipient has no concomitant drug therapies or disease states that limit the use of Symdekoa® and will not be receiving Symdekoa® in combination with any medication that is contraindicated or not recommended per FDA labeling.

**Reauthorization Criteria**

- The recipient continues to meet all initial approval criteria; **AND**
- The prescriber **states on the request** that there is evidence of a positive response to treatment.

**Duration of iInitial and rReauthorization aApproval: 12 months**

---

**References**

Kalydeco (ivacaftor) [package insert]. Boston, MA: Vertex Pharmaceuticals Incorporated; DecemberSeptember 2020. https://pi.vrtx.com/files/uspi_ivacaftor.pdf

Orkambi (lumacaftor/ivacaftor) [package insert]. Boston, MA: Vertex Pharmaceuticals Incorporated; July 2019. https://pi.vrtx.com/files/uspi_lumacaftor_ivacaftor.pdf

Symdeko (tezacaftor/ivacaftor) [package insert]. Boston, MA: Vertex Pharmaceuticals Inc; December 20202019.
~~https://pi.vrtx.com/files/uspi_tezacaftor_ivacaftor.pdf~~https://pi.vrtx.com/files/uspi_tezacaftor_ivacaftor.pdf

| **Revision / Date** | **Implementation Date** |
|---|---|
| Single PDL Implementation | May 2019 |
| Modify age to 6 years or older – Symdeko® / September 2019 | November 2019 |
| Removed Fee-for-Service, modified formatting, added revision table, removed footer, combined all cystic fibrosis agent criteria into one document / December 2020 | January 2020 |
| Formatting changes, updated references / June 2020 | July 2020 |
| Modified age to 4 months for Kalydeco®, updated reference, formatting changes / October 2020 | ~~October 2020~~April 2021 |
| Updated indications for Kalydeco® and Symdeko®, formatting changes, updated references / February 2021 | ~~February~~July 2021 |