Sample Letter of Appeal for Welgemda™ (ribitol)

This resource provides an example of the types of information to consider when submitting a letter of appeal.

When a payer denies a prior authorization (PA) request for Welgemda, your patient has the right to appeal the decision. If they wish to do so, you and your staff may assist by submitting an appeal letter and the requisite supporting documentation to seek to overturn the decision.

Payers may request additional documentation from you to support PA of Welgemda when the initial PA has been denied. The letter of appeal should explain why Welgemda is medically necessary and/or clinically appropriate for the specific patient. It should also include patient-specific information, address the reason(s) for the denials and be signed by the prescriber.

This sample letter is provided for your guidance only. Any letter of appeal should be based on your medical judgment and discretion. Use of any information suggested in this letter does not guarantee that the health plan will approve coverage, and it is not intended to be a substitute for, or an influence on, the independent medical judgment of the healthcare provider. When completing any request, it is the responsibility of the healthcare provider to adhere to the payer's specific requirements at that time.

Reminders for submitting a letter of appeal:

- Ensure the treating physician signs the letter
- Include the appropriate ICD-10-CM diagnosis code
- Include relevant enclosures (refer to pages 6-7 for suggested list)

ICD-10-CM=International Classification of Diseases, 10th Revision, Clinical Modification.

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MAT-US-BBP418-0235 V1

[Physician's letterhead]

[Date]

ATTN: [Health plan contact name]
[Contact title]
[Health plan name]
[Health plan mailing address]
[City, State ZIP]

Subject: **Appeal of Denied Prior Authorization for Welgemda™ (ribitol)**

Patient Name: [Patient's first and last name]
Date of Birth: [MM/DD/YYYY]
Insurance ID/Policy Number: [Insurance ID/policy number]
Insurance Group Number: [Insurance group number]
Claim Number: [Patient claim number]
Diagnosis Code (ICD-10-CM): [G71.036 (FKRP-related LGMD2I/R9)]
Date of Original PA Request: [MM/DD/YYYY]
Date of Denial: [MM/DD/YYYY]
Original PA Reference Number: [Reference number]
Stated Reason for Denial: [Insert reason provided by payer, if available]

Dear [Medical Director/Health Plan Contact Name],

I am writing to formally appeal your denial of prior authorization (PA) for Welgemda™ (ribitol) for my patient, [patient's first and last name], who has a genetically confirmed diagnosis of limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9).

Your stated reason for denial [insert specific denial reason] does not reflect the clinical reality of my patient's condition. As a board-certified [specialist] with [X] years of experience managing patients with neuromuscular disease, your denial decision does not adequately consider the clinical evidence supporting treatment necessity and the irreversible clinical harm of continued delay.

With a genetically confirmed diagnosis of LGMD2I/R9 and no other FDA-approved treatment option available, [patient's name] faces permanent, irreversible, muscle deterioration that cannot be recovered once lost. Each month of denial represents a critical window during which early intervention can prevent irreversible functional decline.

This letter provides my detailed medical rationale for why this denial should be reversed and why Welgemda is medically necessary and appropriate for my patient.

Why Your Denial Is Clinically Inappropriate
Your stated reason for denial, [insert specific denial reason], does not reflect the clinical reality of [patient's name]'s condition and is not medically justified for the following reasons:

1. [Patient's name]'s diagnosis of LGMD2I/R9 is genetically confirmed with *FKRP* mutations documented via [testing method/date]
2. Welgemda is the first and only FDA-approved pharmacologic treatment for LGMD2I/R9 in adult and pediatric patients weighing at least 30 kg. No alternative treatment options are available for my patient
3. [Patient's name] is experiencing progressive functional decline that requires urgent intervention to prevent irreversible harm

The detailed clinical history and evidence supporting each of these points are provided below.

Addressing the Denial Reason
Your denial stated: [Insert specific denial reason from payer].

However, given [patient's name]'s confirmed genetic diagnosis of LGMD2I/R9, the irreversible nature of disease progression, and the absence of alternative FDA-approved treatment options, Welgemda is medically necessary.

I respectfully submit that this rationale is not clinically justified because:

Point 1 [addressing the specific denial criterion]: [Provide specific clinical counter-argument addressing why the denial reason does not apply to this patient's circumstances. For example: if denial is based on "lack of prior treatment trial," explain that Welgemda is the only FDA-approved treatment for LGMD2I/R9. Current standard of care of LGMD2I/R9 is supportive care (physical therapy, assistive devices, respiratory support, etc), which does not slow or stop disease progression. If based on "disease stage," explain why earlier intervention is justified; if based on "limited evidence," cite FORTIFY data; etc].

Point 2 [if applicable, addressing any secondary denial criterion]: [Provide additional clinical justification as needed].

Summary of Patient's Medical History
[Patient's name] is [patient's age] years old and has been in my care since [date], having been referred to me by [referring physician's name] for [reason]. [Patient's name] was originally diagnosed with LGMD2I/R9 on [date].

I have included a synopsis of [his/her/their] clinical history below:

[Complete the following based on your patient's medical history. Remove any content that does not apply to your patient.]

Record of initial diagnosis: [Patient's name]'s diagnosis of LGMD2I/R9 is supported by [summarize diagnostic assessments, including results of neuromuscular examination, genetic testing, serum creatine kinase (CK) testing, electromyography (EMG), and/or muscle MRI]. Records of these evaluations are included in the enclosures, which are listed in full at the end of this letter.

[**Optional explanatory language**, **if applicable to your patient**: The original genetic testing report and complete diagnostic workup may not be readily available in [patient's name]'s current medical record because their diagnosis was established [insert timeframe] and care has since transitioned between providers, institutions, or health systems. As a result, historical genetic records may not have transferred with my patient's current medical chart.]

Supporting assessments: In addition to the primary diagnostic assessments described above, [patient's name]'s diagnosis is supported by [summarize other functional and pulmonary assessments]. Records of these assessments are included in the enclosures, which are listed in full at the end of this letter.

Relevant medical history: [Briefly summarize the patient's medical history. This summary may include LGMD2I/R9-related hospitalizations, LGMD2I/R9-related comorbidities, progressive proximal muscle weakness, frequent falls, supportive care requirements, use of assistive devices, underlying health issues, risk factors, etc]. Relevant documentation is included in the enclosures, which are listed in full at the end of this letter.

LGMD2I/R9 Disease Overview and Rationale for Treatment with Welgemda
LGMD2I/R9 is a rare, progressive, autosomal recessive muscular dystrophy caused by pathogenic mutations in the *FKRP* gene, leading to impaired glycosylation of alpha-dystroglycan (αDG) and progressive muscle fiber damage.[1-2]

In clinical terms, LGMD2I/R9 is a severely life-limiting disease marked by irreversible muscle degeneration and functional deterioration, commonly leading to loss of ambulation, respiratory decline, cardiac dysfunction, and reduced life expectancy.[3-6]

Welgemda is the only FDA-approved therapy indicated for the treatment of LGMD2I/R9 in adult and pediatric patients weighing at least 30 kg.[7] There are no clinically appropriate or formulary alternatives that are FDA-approved pharmacologic treatments for this condition.

To further support the rationale for treatment with Welgemda, I have provided an overview of the FORTIFY phase 3 clinical trial (NCT05775848) and outcomes of the interim analysis.

FORTIFY study overview: The efficacy of Welgemda was established in a phase 3, placebo-controlled, double-blind study in 112 patients with LGMD2I/R9 aged 12-60 years.[8]

FORTIFY efficacy results: At 12 months, Welgemda demonstrated favorable, clinically meaningful improvements across all key outcomes compared with placebo[7,8]:

- **Ambulation** (100-meter timed test [100MTT] velocity): +0.27-m/sec treatment difference vs placebo (*P*<0.0001)
- **Pulmonary function** (forced vital capacity [FVC], sitting): +4.89% predicted treatment difference vs placebo (*P*=0.0071)
- **Serum CK**: −2055 U/L treatment difference vs placebo (*P*<0.0001), indicating reduced muscle damage

- **Muscle biomarker** (glycosylated αDG): +22.78% of control treatment difference at 12 months vs placebo (*P*<0.0001)

Patients in the Welgemda group showed improvement or stabilization in ambulation and pulmonary function, while placebo-treated patients declined. These results were consistent across all prespecified subgroups, including pediatric patients (ages 12-<18) and patients with varying *FKRP* genotypes.[8]

Clinical significance of FORTIFY efficacy results: While the statistical improvements in FORTIFY are compelling, the clinical meaningfulness of these findings for patient outcomes is equally important:

Ambulation and mobility (+0.27 m/sec difference)[8,9]:

- The 100MTT velocity difference of 0.27 m/sec between Welgemda and placebo translates to meaningful preservation of ambulatory function. In real-world terms, this difference can determine whether a patient maintains community ambulation (>1.2 m/sec, capable of independent outdoor mobility) versus limited outdoor mobility (0.8-1.2 m/sec) or becoming housebound (<0.8 m/sec)

Pulmonary function (+4.89% predicted FVC difference)[8,10,11]:

- The ~5% difference in forced vital capacity (FVC) between Welgemda and placebo may appear modest, but it represents the difference between disease stabilization versus progressive respiratory decline

Muscle biomarker and serum CK reduction[8]:

- The ~23% increase in glycosylated αDG, compared with no meaningful change in the placebo group, demonstrates that Welgemda is addressing the underlying molecular defect in LGMD2I/R9. Improved αDG glycosylation may help stabilize muscle fibers, reduce ongoing muscle damage, and support preservation of muscle function over time
- The rapid and sustained reduction in serum CK (from baseline 2449 U/L to 410 U/L at 12 months in Welgemda-treated patients indicates active reduction of ongoing muscle damage

FORTIFY safety results[8]:

- Welgemda was generally well tolerated, with a safety profile comparable to placebo
    - Grade ≥3 treatment-emergent adverse events (TEAEs) occurred at similar rates between groups (5.4% for Welgemda vs 5.3% for placebo)
    - Serious TEAEs were reported in 5.4% of Welgemda-treated individuals compared to 7.9% of those receiving placebo. No treatment-related serious TEAEs were reported in either arm, and no deaths occurred during the study
- The most common TEAEs (≥10% in the Welgemda arm) were diarrhea (39.2% vs 52.6% for placebo), procedural pain (20.3% vs 10.5%), fall (18.9% vs 26.3%), nasopharyngitis (17.6% vs 21.1%), nausea (16.2% vs 7.9%), arthralgia (14.9% vs 10.5%), headache (10.8% vs 13.2%), COVID-19 (10.8% vs 13.2%), and influenza (10.8% vs 7.9%)
- All events of diarrhea were mild to moderate (Grade 1-2) and no serious TEAEs related to diarrhea were reported

Requested Therapy

I have prescribed my patient a maintenance dose of Welgemda [9 g or 12 g] packets for oral solution to be taken [directions]. Based on my patient's weight, this is within the recommended dosing guidelines of [appropriate FDA-approved dosing schedule] twice daily for the treatment of LGMD2I/R9.

For prescriber reference, the FDA-approved dosing schedule for Welgemda is as follows:

- Patients weighing >50 kg: 12 g orally twice daily
- Patients weighing ≥30 kg and ≤50 kg: 9 g orally twice daily

Summary

Based on the clinical evidence and facts presented above, your denial of prior authorization for Welgemda is not medically justified. [Patient's name] has a confirmed diagnosis of LGMD2I/R9, is experiencing progressive functional decline, and is an appropriate candidate for treatment with Welgemda—the only FDA-approved therapy for this condition. My professional opinion is that failure to provide timely access to Welgemda will cause [patient's name] to face continued irreversible deterioration of [summarize disease-related risks associated with non-treatment, potentially including muscle function, pulmonary function, and/or loss of ambulation].

Considering that no alternative treatment option is currently available and given the shortened life expectancy and irreversible disease progression associated with LGMD2I/R9, denial or delay of Welgemda treatment represents an active clinical harm with permanent consequences for my patient. Therefore, I respectfully request that you reverse your denial and provide prior authorization for Welgemda for [patient's name] and expedite your review given the time-sensitive nature of my patient's condition and the progressive nature of LGMD2I/R9.

Please contact my office at [phone number, fax number, and/or email] if additional information is required. I am available for a peer-to-peer review at your request and welcome the opportunity to discuss my patient's case directly. I look forward to your prompt response.

Sincerely,
[Optional: Physician signature]
[Physician typed name and credentials]
[NPI Number]
[Physician contact information]

Enclosures

[List enclosures, which may include:

- Welgemda Prescribing Information
- Clinical notes/medical records, including:
    - Date of initial diagnosis and diagnostic test results (such as genetic testing report confirming *FKRP* mutation[s], CK testing, EMG, and/or muscle MRI)
    - Supporting functional assessment results (such as 10-meter walk test, North Star Assessment for Limb Girdle-type Dystrophies, FVC, etc)

    - Supportive care requirements, assistive devices, underlying health issues, etc, if applicable
- Additional supporting clinical documentation, including patient weight, if applicable
- Relevant peer-reviewed articles and supportive literature]

CK=creatine kinase; FDA=US Food and Drug Administration; FKRP=fukutin-related protein.

References: 1. Liu W, Pajusalu S, Lake NJ, et al. Estimating prevalence for limb-girdle muscular dystrophy based on public sequencing databases. *Genet Med*. 2019;21(11):2512-2520. doi:10.1038/s41436-019-0544-8 **2.** Bouchard C, Tremblay JP. Limb-girdle muscular dystrophies classification and therapies. *J Clin Med*. 2023;12(14):4769. doi:10.3390/jcm12144769 **3.** Miller CL, Coffey LN, Mockley, SRH, et al. Predicting loss of ambulation in limb girdle muscular dystrophy R9. *Ann Clin Transl Neurol*. 2026;13(6):1154-1159. doi:10.1002/acn3.70299. **4.** Jensen SM, Müller KI, Mellgren SI, et al. Epidemiology and natural history in 101 subjects with FKRP-related limb-girdle muscular dystrophy R9. The Norwegian LGMDR9 cohort study (2020). *Neuromuscul Disord*. 2023;33(2):119-132. doi:10.1016/j.nmd.2022.11.005 **5.** Libell EM, Richardson JA, Lutz KL, et al. Cardiomyopathy in limb girdle muscular dystrophy R9, FKRP related. *Muscle Nerve*. 2020;62(5):626-632. doi:10.1002/mus.27052 **6.** Villa C, Sun L, Bartley K, et al. Long-term survival, quality-adjusted life-years, and economic burden in limb-girdle muscular dystrophy type 2I/R9: a health outcomes model. Poster presented at: Muscular Dystrophy Association (MDA) Clinical & Scientific Conference; March 8-11, 2026; Orlando, Florida. **7.** Welgemda. Prescribing Information. BridgeBio Pharma, Inc.; 2026. **8.** Mathews KD, Sproule D, Lee A, et al. Interim analysis from ongoing Phase 3 FORTIFY study of BBP-418 for patients with limb girdle muscular dystrophy 2I/R9 meets efficacy endpoints. Poster presented at: Muscular Dystrophy Association (MDA) Clinical & Scientific Conference; March 11, 2026; Orlando, FL. **9.** Alfano LN, James MK, Peterson KG, et al. Prospective observational study of FKRP-related limb-girdle muscular dystrophy R9: a GRASP consortium study. *Ann Clin Transl Neurol*. 2025;12(2):332-344. doi:10.1002/acn3.52276 **10.** CardiacDirect. Normal values for a spirometry test. May 13, 2025. Accessed August 11, 2026. https://www.cardiacdirect.com/normal-values-for-a-spirometry-test/ **11.** Gedlinske AM, Stephan CM, Mockler SRH, et al. Motor outcome measures in patients with FKRP mutations: a longitudinal follow-up. *Neurology*. 2020;95:e2131-e2139. doi:10.1212/WNL.0000000000010604