Aligning product communications and medical information for rare-disease programmes

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Abstract

Rare-disease programmes face two tightly linked operational challenges:

1. Complex, country-by-country compliance requirements for product communications

2. Highly variable, individualised medical information needs from a diverse set of inquirers, including patients, caregivers, general practitioners (GPs), key opinion leaders (KOLs) and patient organisations

These challenges share a single dependency: reliable, up-to-date source content governed by clear processes. Without a joint approach, sponsors risk inconsistent messaging, delayed local approvals, regulatory exposure and poor inquirer experience.

This whitepaper offers practical advice to help you align a single master source-of-truth for product communications with an inquirer-centric medical information function.

Introduction

Rare diseases amplify the normal complexities of pharma communications due to small, geographically dispersed patient populations, limited natural history data and specialist clinical expertise concentrated in a few centres.

Sponsors must, therefore, deliver legally compliant, locally appropriate communications while also answering a wide range of individual questions — from straightforward product administration to nuanced clinical interpretation.

Because both compliance and medical information depend on the same authoritative content and localisation processes, they must be governed together to ensure safety, consistency and timeliness.

The UK/EU compliance landscape for product communications

Understanding the regulatory landscape is the first operational step; the right processes make local complexity manageable.

The UK and EU present a fragmented environment where regional frameworks like the EFPIA (European Federation of Pharmaceutical Industries and Associations) sit alongside country-specific codes, vetting routines and archive requirements.

Recognising both the shared principles and the unique local obligations is essential to avoid delays, inconsistencies and regulatory risk. As such, sponsors must design processes that recognise those differences rather than assume a single English asset will work everywhere.

Core challenges

- **Country-by-country variability.** Although the EFPIA offers a regional baseline, national interpretation and practice vary widely. Assets that pass in one market frequently require adaptation, pre-vetting or rejection in another — for example, formal pre-vetting in Germany, restrictions on patient imagery in Denmark and local archive/reporting obligations in Norway.

- **Non-promotional materials also draw scrutiny.** Review is not limited to promotional adverts: educational content, patient-support programme materials, early-access communications and interactions with healthcare professionals and healthcare or patient organisations commonly trigger local verification, certification or sign-off requirements.

- **Small sponsor scalability constraints.** Large companies often manage local review through in-country teams; small and mid-sized biotechs typically do not, yet they face the same legal, ethical and GDPR obligations. Rare-disease programmes — with few patients spread across many jurisdictions — make this scalability problem acute.

- **Regulatory and reputational risk.** Non-compliance can lead to corrective actions, fines, reputational damage and, in extreme cases, limitations on commercial activities. Because rare-disease communities are small and highly engaged, even a single communications misstep can damage trust with patients, clinicians and patient organisations.

Practical responses

- **Create a most-stringent master communications document.** Draft a single source-of-truth to the highest applicable national standard so that every local derivative is traceable back to an auditable master. This reduces rework and ensures consistency between promotional, non-promotional and medical information materials.

- **Define a controlled local-adaptation workflow.** Treat adaptation, translation and revalidation as formal steps: map country rules, appoint named local approvers, set timelines for pre-vetting where required, and record signoffs and version history. Translations and cultural adaptations must be reviewable compliance steps

- **Use a managed local-approver network or centralised provider.** Where in-country teams are absent, engage trusted local reviewers or a pan-European service partner with local signatories to share accountability and surface national issues early, adjusting the nuanced differences between approved product information across different countries. This is particularly valuable for small sponsors.

- **Embed GDPR and ethics checks into approvals.** Review patient-facing activities and patient organisation engagements for data privacy and ethical sensitivity as part of every local validation, and document consent/processing arrangements where required.

- **Pre-approve medical information snippets and fast-track routes for routine queries.** Maintain a library of country-validated, tiered snippets so that teams can reply quickly without ad hoc full reviews; create a formal fast-track approval for time-sensitive, non-promotional responses.

- **Maintain robust archiving and audit trails.** Keep searchable records of all local variants, approvals and translations to demonstrate compliance during audits or enquiries.

- **Operationalise cross-functional governance.** Convene regulatory and medical representatives on a regular basis to resolve localisation disputes, prioritise adaptations and ensure trends feed back into the master document for revision.

Adopting these responses will reduce the likelihood of inconsistent messaging, speed local publication cycles and protect patient trust — all essential for safe, effective communications in rare-disease programmes.

Quick compliance checklist

- ☐ Create a **master product communications document** drafted to the most stringent applicable national code.

- ☐ Identify target countries and map local rules (vetting, archive, image restrictions).

- ☐ Build a **local approver directory** with signatory authority per country.

- ☐ Define the **adaptation ➜ translation ➜ revalidation** workflow and timelines.

- ☐ Assess GDPR/data-handling requirements for patient-facing activities.

- ☐ Decide between in-house local reviewers vs a centralised service partner and document co-accountability.

- ☐ Maintain version control and an audit trail of approvals for every local variant.

Why an inquirer-centric medical information model matters in rare disease

KOLs require depth and nuance, GPs need clear referral guidance, and patients/caregivers require practical, empathetic information. Delivering the right answer depends on identifying the inquirer and adapting the same factual core into different tones and levels of detail. Medical information must, therefore, be both scientifically rigorous and humanised.

Good medical information transforms facts into useful actions for different audiences; in rare diseases, that means multiple tiers of technical depth and empathetic language.

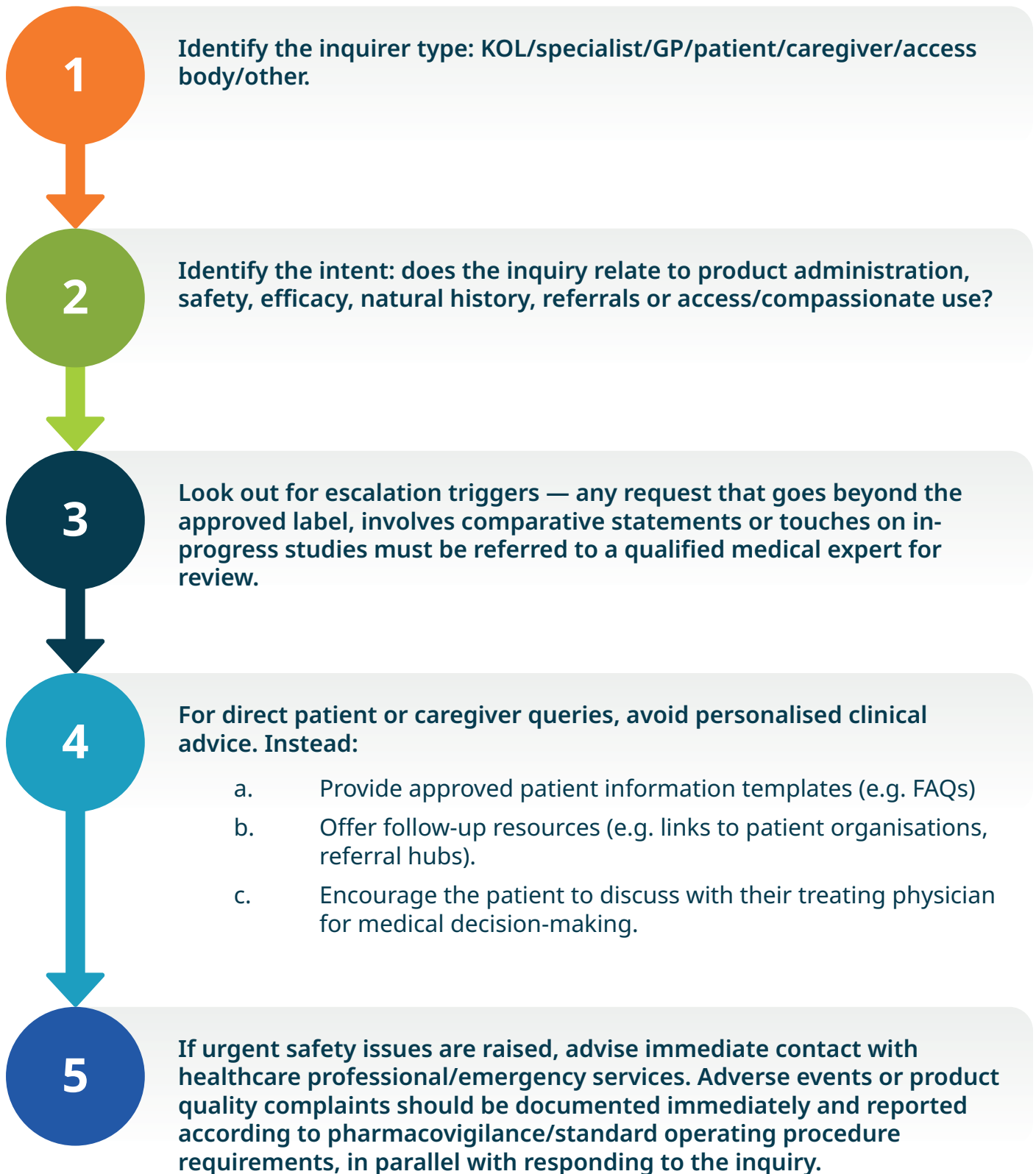## Operational constraints and enablers

- **Language and literacy variability.** Many inquiries arrive in English, but responses must often be provided in other languages and at various literacy levels, so plan for human and technological translation support.

- **Geographic dispersion and scale limitations.** Centralised expertise supported by technology and selected local partners is typically the most pragmatic medical information model for rare diseases.

- **24/7 access and partnership needs.** Continuous coverage and links to patient access services often require strategic vendor partnerships for small sponsors.

Best practices for an inquirer-centric approach

- **Segment stakeholders and tailor responses.** Prepare response templates at multiple technical levels (KOLs, healthcare professionals, GP, patient/caregiver) and train triage to identify the inquirer type quickly.

- **Escalate to specialised medical experts when needed.** Route complex interpretive questions to qualified medical experts with clear escalation rules to preserve compliance.

- **Implement feedback loops to refine content.** Use inquiry trends to reveal knowledge gaps and update the master content accordingly.

- **Use technology as a connector, not a replacement.** Systems should route inquiries, manage multilingual replies and log interactions — but human clinical judgement remains central.

Quick triage rules (first 10–30 seconds):

The first moments of any inbound request set the tone for compliance, efficiency and trust. A structured triage framework helps medical information teams quickly determine who is asking, what they need and how best to respond.

1 — **Identify the inquirer type: KOL/specialist/GP/patient/caregiver/access body/other.**

2 — **Identify the intent: does the inquiry relate to product administration, safety, efficacy, natural history, referrals or access/compassionate use?**

3 — **Look out for escalation triggers — any request that goes beyond the approved label, involves comparative statements or touches on in-progress studies must be referred to a qualified medical expert for review.**

4 — **For direct patient or caregiver queries, avoid personalised clinical advice. Instead:**

    a. Provide approved patient information templates (e.g. FAQs)

    b. Offer follow-up resources (e.g. links to patient organisations, referral hubs).

    c. Encourage the patient to discuss with their treating physician for medical decision-making.

5 — **If urgent safety issues are raised, advise immediate contact with healthcare professional/emergency services. Adverse events or product quality complaints should be documented immediately and reported according to pharmacovigilance/standard operating procedure requirements, in parallel with responding to the inquiry.**

Where product communication compliance and medical information overlap

Mapping where these two disciplines meet makes it easier to design joined processes that reduce friction and risk:

- **Single source of truth.** Both functions need the same updated master product information as inconsistencies propagate risk.

- **Localisation and translation.** Adaptations affect product communications and medical information answers — a mistranslation can create regulatory and safety issues.

- **Interactions with patient organisations.** Engagements with patient organisations pose both ethical and GDPR considerations for communications and medical information.

- **Timeliness vs approval governance.** The need for speed in medical information can clash with formal local review cycles; without pre-approved snippets or a fast-track, delays or risky ad hoc replies may occur.

To avoid these friction points, sponsors should make the master product communications document the single source of truth, governed by a cross-functional committee, and use it to produce pre-approved, tiered medical information snippets and a controlled local-adaptation process. This alignment reduces compliance risk and improves speed and quality of answers to every inquirer.

About TMC

TMC is a strategic partner to small and mid-sized biotech companies, delivering integrated solutions across the full product lifecycle — from early development to market access and commercialisation. With deep expertise in regulatory, clinical, medical, pharmacovigilance and quality specialties, we help biotechs navigate complexity, accelerate timelines and optimise outcomes.

Email us directly at **connect@tmcpharma.com** to find out how we can help you ensure compliant product communications and implement an inquirer-centric approach to medical information through our combined medical services.

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