Local burden data collection framework

Introduction

Understanding the local burden of amyloidosis is an essential step in advocating for improved services and establishing specialized centers. Although the global impact of amyloidosis is increasingly recognized, local data are often limited or fragmented.

This guide has been developed to support healthcare professionals, clinical leaders, and administrators in systematically gathering and using local data to strengthen the case for amyloidosis services. By capturing information on disease prevalence, diagnostic delays, referral patterns, patient outcomes, and healthcare resource utilization, stakeholders can better illustrate the clinical and economic burden of amyloidosis in their healthcare system.

This framework provides practical, structured guidance on how to collect, analyze, and present local data on amyloidosis. It outlines step-by-step approaches for translating data into evidence-based arguments that highlight unmet needs and support requests for resources. In addition to methodological guidance, this document includes examples of data collection tools and templates that can be adapted to different healthcare settings.

The framework at a glance

Phase	You will learn how to...	Outcome
1. PLAN	Define your objectives and identify available data sources	Create a data collection plan
2. COLLECT	Gather clinical-, system-, and patient-level data	Generate raw data
3. ANALYZE	Identify trends, existing resources/demands, gaps, and key messages	Build summary tables and visuals of key data
4. TRANSLATE	Convert findings into arguments for funding, advocacy, and resource allocation	Formulate key messages for advocacy
5. PRESENT	Communicate data to decision-makers, including hospital leadership, health authorities, and funding bodies	Present your findings in the form of a presentation or report document

STEP 1 - PLAN: Planning your local data collection

Careful planning helps to ensure that the data you collect are relevant and feasible for demonstrating the local burden of amyloidosis. At this stage, define what you want to measure, identify available data sources, consider governance requirements, and assign responsibilities within your team.

1. Define your objectives

Start by identifying the key questions that your data collection should address. Clear objectives will guide what data are needed and how they will be used. Objectives should be specific and focused on generating evidence that can support service development. Examples may include:

- Quantifying diagnostic delays to support the development of faster referral pathways
- Estimating the number of patients diagnosed or treated locally to demonstrate service demand
- Identifying referral patterns and points of delay in the diagnostic pathway
- Assessing disease stage at diagnosis or patient outcomes
- Evaluating healthcare resource utilization, such as hospitalizations or specialist visits

2. Identify available data sources

Consider which data sources are accessible within your institution or region. Mapping available sources early helps to determine what data can be realistically collected. Relevant information may already exist but be distributed across multiple systems. Common sources include:

- Hospital records, electronic health records, and laboratory databases
- National or international registry data, where available
- Clinical audits and departmental records
- Physician or patient surveys

AstraZeneca has provided an educational grant towards this independent program. This program is intended for healthcare professionals only.

The table below provides an overview of the types of metrics you could measure, along with possible sources for these data.

Type of burden	Metrics	Data sources
Clinical	• Incidence and prevalence of amyloidosis cases diagnosed locally • Distribution by amyloidosis subtype (AL, ATTR, AA, etc.) • Time from symptom onset to diagnosis (median and range), time from diagnosis to start of disease-modifying therapy • Organ systems involved at presentation • Mortality and morbidity data (if available)	• Electronic health records • Laboratory or pathology reports and databases • Pathology reports • Referral logs • Clinical audits or department records • National or international registries
System	• Number of departments involved in diagnosis and management • Number of diagnostic tests performed (e.g., biopsies, imaging, mass spectrometry) • Referral pathways and waiting times • Missed or delayed diagnoses (identified retrospectively)	• Departmental records • Multidisciplinary meeting logs • Referral data • Laboratory or pathology reports and databases • National or international registries
Economic	• Mean number of hospitalizations before diagnosis • Cost of unnecessary investigations or misdirected referrals • Cost of managing advanced organ involvement vs early-stage disease	• Departmental budget data • Hospital finance records • Claims data
Humanistic	• Patient-reported delays and misdiagnoses • Quality of life impact (fatigue, pain, work absence) • Emotional and social impact on patients and caregivers	• Short patient surveys, patient association/advocacy data • Literature benchmarks

3. Examine ethical and governance considerations

Ensure that all data collection activities comply with local governance and data protection requirements. This may include:

- Obtaining ethical or institutional approvals, where required
- Adhering to principles of informed consent
- Ensuring patient confidentiality and data anonymization
- Clarifying data ownership and sharing agreements
- Complying with local data protection regulations

4. Align on roles and responsibilities

Decide who will collect, verify, and present data. This can be adapted depending on resource in your institution; typical roles could include:

- Clinician champion: Leads the initiative and defines objectives
- Data analyst or data manager: Extracts and analyzes data
- Nurse or clinical coordinator: Supports data collection and coordination
- Administrative support: Assists with documentation and reporting

STEP 2 - COLLECT: Gathering your data

This stage focuses on systematically gathering the data needed to demonstrate the local burden of amyloidosis. A structured approach helps to ensure the consistency, completeness, and usability of the data.

1. Define the target population

Clearly define which patients will be included in your analysis. For example:

- Patients with confirmed amyloidosis diagnosed within a specified time frame (e.g., in the past 3–5 years)

2. Extract core variables

Identify and extract key variables aligned with your objectives. For example:

- Patient demographics (age, sex)
- Time from symptom onset to diagnosis
- Time from diagnosis to treatment initiation
- Number of referrals or specialists seen before diagnosis
- Disease characteristics (e.g., subtype, organ involvement)

3. Collect contextual institutional data

Supplement patient-level data with information about your local healthcare system. For example:

- Diagnostic resources available (e.g., access to mass spectrometry, genetic testing)
- Number of referring healthcare providers, clinics, and hospitals
- Current structure of referral pathways or multidisciplinary care
- This context helps to explain observed gaps and supports stronger advocacy messages.

4. Collect qualitative input

Where possible, gather insights from healthcare professionals and patients at your institution. For example:

- Physician surveys (e.g., referral patterns, diagnostic challenges, awareness)
- Patient surveys (e.g., delays, misdiagnoses, quality of life impact)
- Ensure that all data collection complies with local governance and data sharing requirements.

5. Validate and clean data

Review and prepare your dataset before analysis:

- Cross-check data across sources and departments, where possible
- Remove duplicates and incomplete records
- Standardize variables (e.g., date formats, definitions)
- Generate basic summary statistics (e.g., median, range, frequency)

STEP 3 - ANALYZE: Analyzing and interpreting your data

The goal of this stage is to convert raw data into clear, meaningful insights that highlight unmet needs.

1. Quantitative analysis

- Use simple descriptive measures
- Use mean, median, and range for continuous variables
- Use percentages for categorical variables

Example outputs:

- Median time to diagnosis: 18 months
- Patients seeing >3 physicians before diagnosis: 65%
- Hospitalizations before diagnosis: 2.3 per patient
- Median time to treatment: 21 months

2. Comparative analysis

Contextualize your findings by comparing them with published data or registry benchmarks, e.g.:

Indicator	Local result	Benchmark	Interpretation
Diagnostic delay	18 months	12 months (literature)	Delay longer than average → opportunity for pathway improvement

STEP 4 - TRANSLATE: Translating data into advocacy messages

This stage focuses on turning data into clear, action-oriented messages that support the development of amyloidosis services.

1. Use a data-to-message framework

You can use a data-to-message framework to structure your findings into four sections:

- Fact: What does the data show?
- Impact: Why does it matter clinically or systemically?
- Solution: What change is needed?
- Call to action: What specific action or resource is required?

The example below illustrates how the data-to-message framework can be used in practice. You can use the message builder template at the end of this document to create messages from your findings.

Finding	Implication	Solution	Call to action
18-month diagnostic delay	Late detection leads to irreversible organ damage and poorer treatment outcomes	Establish dedicated diagnostic pathway	Support funding for multidisciplinary clinic

2. Tailor messages to your audience

Tailor your messages to ensure relevance for each stakeholder, for example:

Audience	Focus	Example message
Hospital leadership	Efficiency, outcomes, institutional prestige, eventual cost savings	"A specialized amyloidosis center would reduce repeated hospitalizations and could position our hospital as a national/regional leader."
Policymakers	Health equity, alignment with rare disease strategies	"Patients in our region experience diagnostic delays that are nearly twice the national average. Targeted investment could help to close this gap."
Patient groups	Awareness/partnerships	"We want to collaborate to improve early diagnosis and quality of life for patients living with amyloidosis."

STEP 5 - PRESENT: Presenting and communicating your findings

Effectively presenting your data is critical to achieving impact and securing support.

1. Structure your report or presentation

- Executive summary: Create a 1-page overview of key findings and recommendations
- Methods: Briefly describe data sources and time frame
- Key findings: Include visuals and tables
- Implications: Explain why the findings matter
- Recommendations: Set out actionable next steps

2. Prepare for discussion

Be ready to answer questions, such as:

- What are the main barriers to timely diagnosis?
- Where are the gaps in the current pathway
- What resources would improve patient outcomes?
- How would funding for an amyloidosis service help this hospital?
- Why would an amyloidosis service be a valuable use of resources?

3. Apply visual storytelling principles

When generating slides for presentation:

- Use 1 or 2 key charts or statistics per slide
- Highlight important numbers clearly
- Use patient stories or quotes where appropriate to humanize data
- End every presentation with a specific "ask" or call to action (e.g., "Support the appointment of an amyloidosis nurse coordinator.")

4. Sustaining data collection

Ongoing data collection is important to track progress and support continued investment. You could:

- Establish a simple local registry, where feasible
- Monitor key indicators regularly (e.g., quarterly), such as:
- Number of new diagnoses
- Time to diagnosis
- Number of multidisciplinary team meetings

Summary and templates

This framework provides you with a structured, step-by-step approach to help you collect and use local data to support the development of amyloidosis services in your setting. By following this process, you can move from limited or fragmented data to clear, evidence-based insights that demonstrate unmet need and encourage action.

To support you in this process, a set of practical templates is provided on the following pages. These are designed to help you get started and can be adapted to your local context.

- **Physician survey template:** You can disseminate this survey among clinicians in your department, hospital, and among other local referring healthcare providers (e.g., primary care) to understand referral patterns, diagnostic challenges, and awareness of amyloidosis.
- **Patient survey template:** You can disseminate this survey among patients to capture patient experience and quality of life impact.
- **Message builder worksheet:** You can use this template to translate data into clear and action-oriented advocacy messages.

Physician survey form: Understanding local amyloidosis burden

This survey aims to better understand the barriers faced my clinicians and healthcare services treating patients with amyloidosis. Your responses will help to inform the development of services to support amyloidosis diagnosis and management.

Section 1: Respondent information

1a. Specialty:

1b. Years in practice:

☐ <5 ☐ 5–10 ☐ >10

1c. Practice setting:

☐ Academic ☐ Community

☐ Other:

1d. Type of practice:

☐ Tertiary hospital ☐ Secondary hospital ☐ Primary care ☐ Private practice

☐ Other:

Section 2: Awareness and experience

2a. How familiar are you with amyloidosis?

☐ Not familiar ☐ Somewhat familiar ☐ Very familiar

2b. Approximately how many patients with suspected or confirmed amyloidosis have you seen in the past 2 years?

☐ 0 ☐ 1–5 ☐ 6–10 ☐ >10

Section 3: Referral and diagnosis

3a. Do you have access to a multidisciplinary team for complex cases?

☐ Yes ☐ No ☐ Unsure

3b. What is your typical action when you suspect amyloidosis?

☐ Refer to specialist ☐ Order tests ☐ Monitor

☐ Other:

3c. Which specialties do you typically refer patients to?

☐ Cardiology ☐ Hematology ☐ Neurology ☐ Nephrology

☐ Other:

3d. In your experience, what are the main barriers to timely diagnosis? (Select all that apply)

☐ Lack of awareness ☐ Nonspecific symptoms ☐ Limited access to diagnostic tests

☐ Unclear referral pathways

☐ Other:

3e. In your experience, what is the estimated time from first symptoms to diagnosis?

☐ <6 months ☐ 6–12 months ☐ 1–2 years ☐ >2 years

Section 4: System challenges

4a. Are clear referral pathways for amyloidosis available in your setting?

☐ Yes ☐ No ☐ Unsure

4b. What factors influence your referral decision? (Select all that apply)

☐ Availability of specialists ☐ Distance to center ☐ Waiting times ☐ Awareness of referral pathways

☐ Other:

4c. Which resources are currently limited in your healthcare setting? (Select all that apply)

☐ Diagnostic tools ☐ Specialist access ☐ Multidisciplinary care ☐ Patient education resources

☐ Other:

Section 5: Opportunities for improvement

5a. What would most improve early diagnosis in your setting?

5b. Would you support the development of a specialized amyloidosis center?

☐ Yes ☐ No ☐ Unsure

Patient survey form: Understanding the amyloidosis journey

This survey aims to better understand the patient experience of amyloidosis, including the journey to diagnosis, challenges faced, and impact on daily life. Your responses will help to improve awareness, diagnosis, and care pathways.

Section 1: About you

1a. Age group:

☐ <40 ☐ 40–60 ☐ >60

1b. Sex (optional):

☐ Female ☐ Male ☐ Prefer not to say ☐ Other: _____

1c. Region: _____

1d. Type of amyloidosis (if known):

☐ AL ☐ ATTR ☐ AA ☐ Unsure ☐ Other: _____

Section 2: Your diagnostic journey

2a. What symptoms did you first experience? (Select all that apply)

☐ Fatigue ☐ Shortness of breath ☐ Swelling (e.g., legs, abdomen) ☐ Numbness or tingling ☐ Weight loss

☐ Other: _____

2b. Approximately how long did it take to receive a diagnosis from when your symptoms first started?

☐ <6 months ☐ 6–12 months ☐ 1–2 years ☐ >2 years

2c. How many healthcare professionals did you see before receiving a diagnosis?

☐ 1–2 ☐ 3–4 ☐ 5 or more

2d. Were you initially given a different diagnosis (misdiagnosed)?

☐ Yes ☐ No ☐ Unsure

If yes, please specify (optional): _____

Section 3: Experience of care

3a. Did you experience delays in receiving your diagnosis?

☐ Yes ☐ No ☐ Unsure

3b. What were the main challenges you experienced during the diagnostic process? (Select all that apply)

☐ Lack of awareness among healthcare professionals ☐ Symptoms not recognized as serious

☐ Delays in referrals ☐ Limited access to tests ☐ Long waiting times

☐ Other: _____

3d. How would you rate your overall experience of the diagnostic process?

☐ Good ☐ Fair ☐ Poor

Section 4: Impact on your life

4a. Which aspects of your life have been affected by amyloidosis? (Select all that apply)

☐ Physical health ☐ Ability to work ☐ Daily activities ☐ Emotional well-being ☐ Social life

☐ Other: _____

4b. Overall, what impact has amyloidosis had on your quality of life?

☐ Mild ☐ Moderate ☐ Severe

4c. Has your condition affected your ability to work or carry out usual activities?

☐ Yes ☐ No

Section 5: Improving care

5a. What do you think would have improved your experience of diagnosis and care?

_____

5b. What is the most important change needed to improve care for people with amyloidosis in your region?

_____

5c. Would you support the development of a specialized amyloidosis center in your region?

☐ Yes ☐ No ☐ Unsure

Message builder worksheet

Use this template to translate data into clear and action-oriented advocacy messages. An example has been provided in the top row.

Finding	Implication	Solution	Call to action
18-month diagnostic delay	Late detection leads to irreversible organ damage and poorer treatment outcomes	Establish dedicated diagnostic pathway	Support funding for multidisciplinary clinic