From Nightingale to Now: Why Visualisations Are Still Essential in The Statisticians' Toolkit

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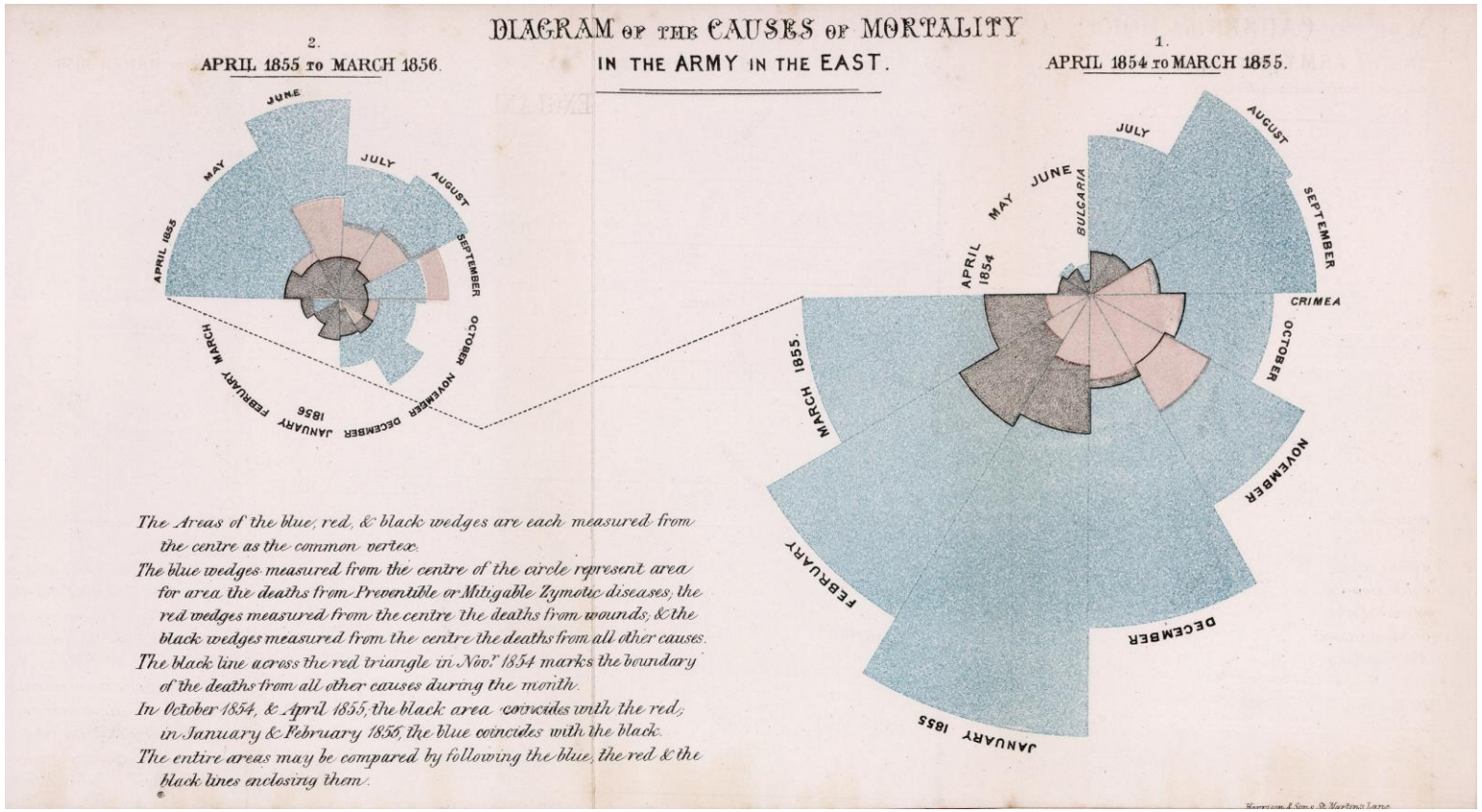
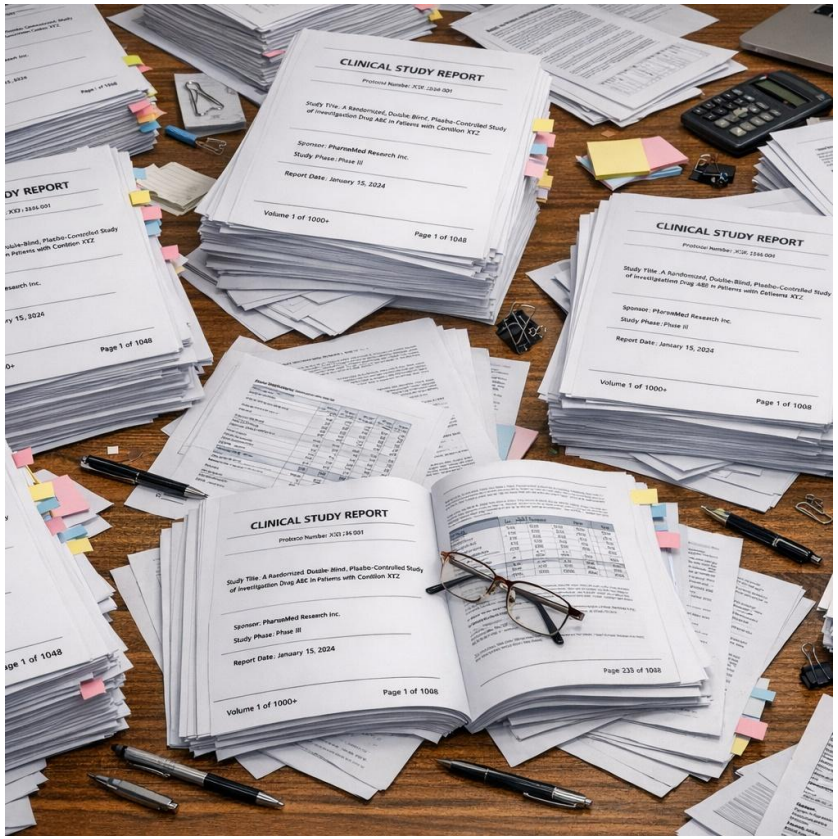
Acknowledgment:
Any reference to disease area has been removed

Thank you to:
Trevor Smart and the project team

From Nightingale to Now …





Florence Nightingale, 1857

Visualisations are still important!!!

Background

Ultra-rare genetic disease (~600 cases recorded worldwide)

Causes developmental delay

Paediatric patients

Data extracted from medical records of 62 patients

No standard protocol for how data were collected

The problem

A typical clinical dataset

Patient ID	Gender	Timepoint	Endpoint 1	Endpoint 2	Measure 1	Measure 2
1	Female	1 year	Positive	Stable	78	120 mg/dL
1	Female	2 years	Positive	Declined	84	150 mg/dL
1	Female	3 years	Negative	Stable	76	180 mg/dL
2	Male	1 year	Positive	Improved		90 mg/dL
2	Male	2 years	Negative	Stable	93	110 mg/dL
...	...	...	...	...	...	...

Our dataset

- Different endpoints
- Measured at different times
- High missingness

Patient ID	Gender	Timepoint	Endpoint 1	Endpoint 2	Another Endpoint	A Different Endpoint
1	Female	5.3 years	Positive			
1	Female	6 years		Negative		
1	Female	6.1 years		Positive		
2	Male	1 year				Negative
2	Male	1.2 years			Negative	
...	...	...	...	...	...	...

Background

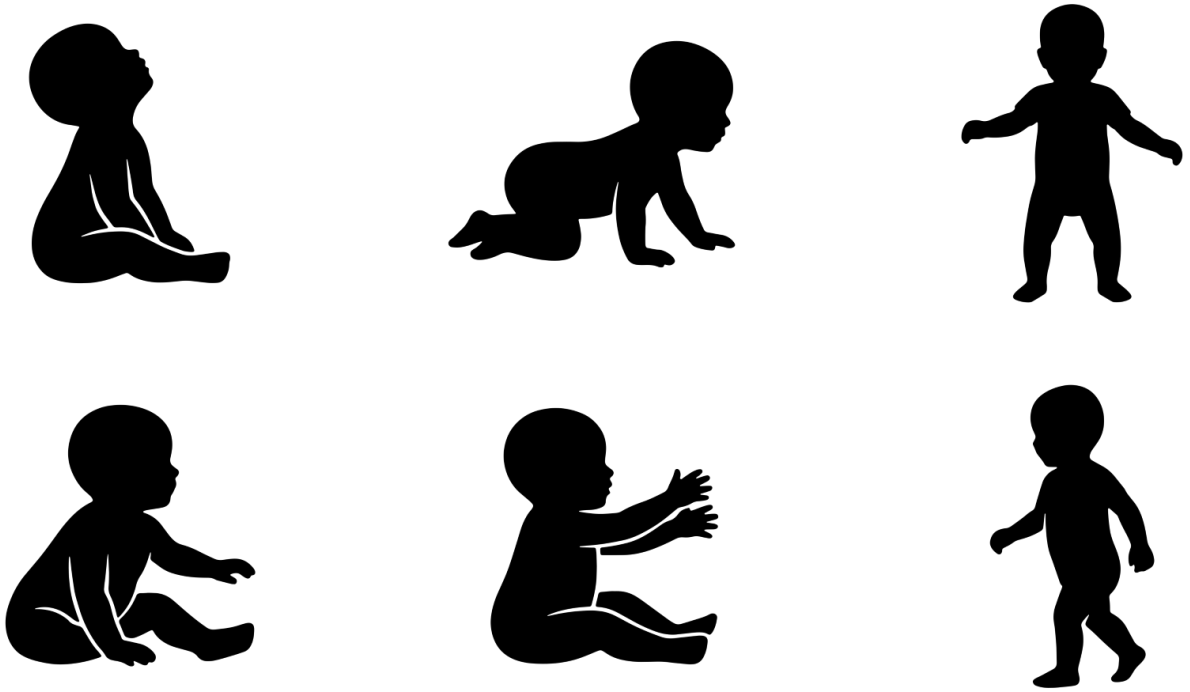
Ultra-rare genetic disease (~600 cases recorded worldwide)

→ limited data

→ Data are sparse, messy and inconsistent

We can’t model so we visualised the data

Developmental Milestones



Developmental Milestones

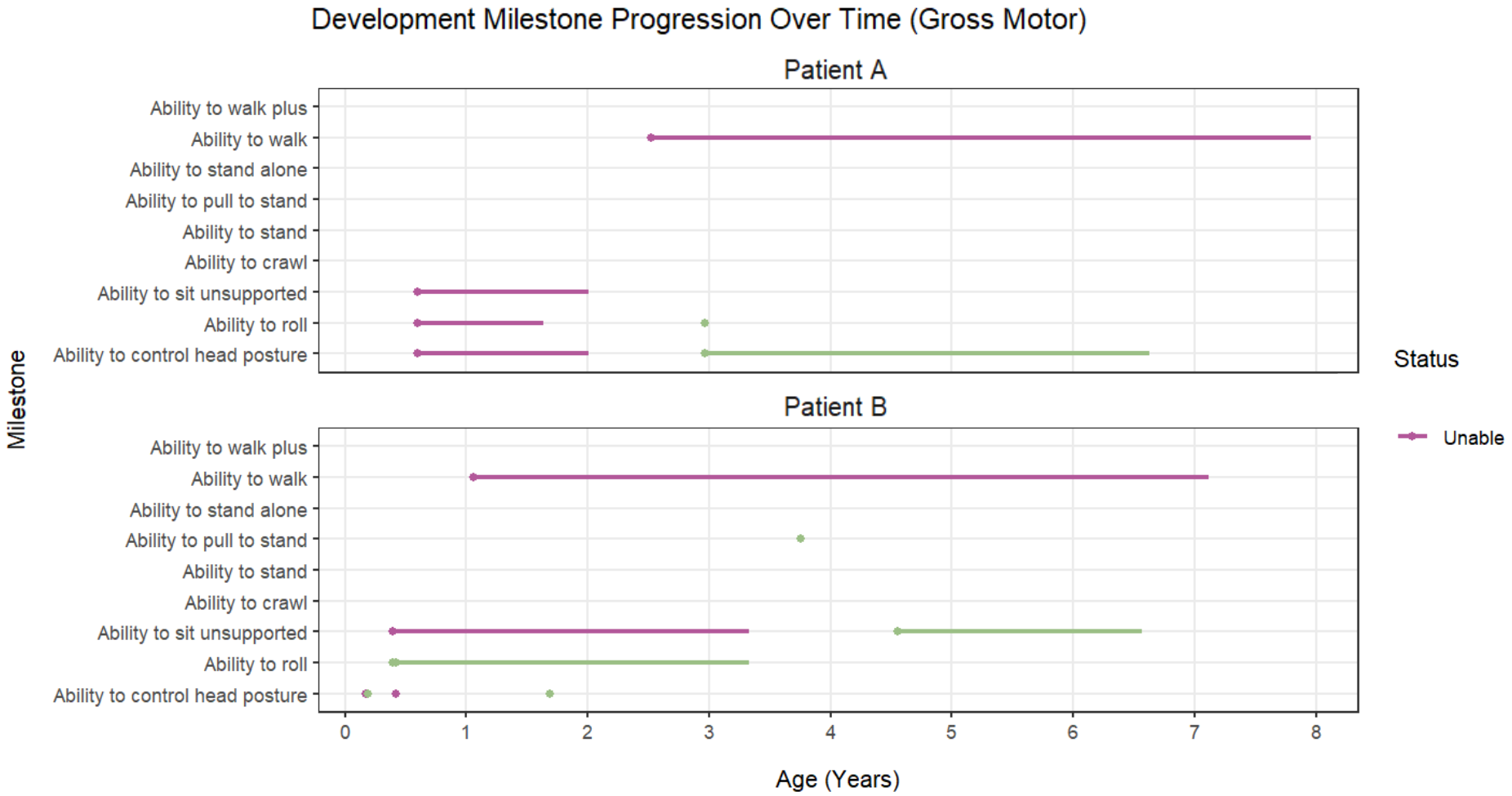
Getting a more complete picture

1. Add groupings

Developmental Milestones

Getting a more complete picture

1. Add groupings
2. Add ordering



Developmental Milestones

Getting a more complete picture

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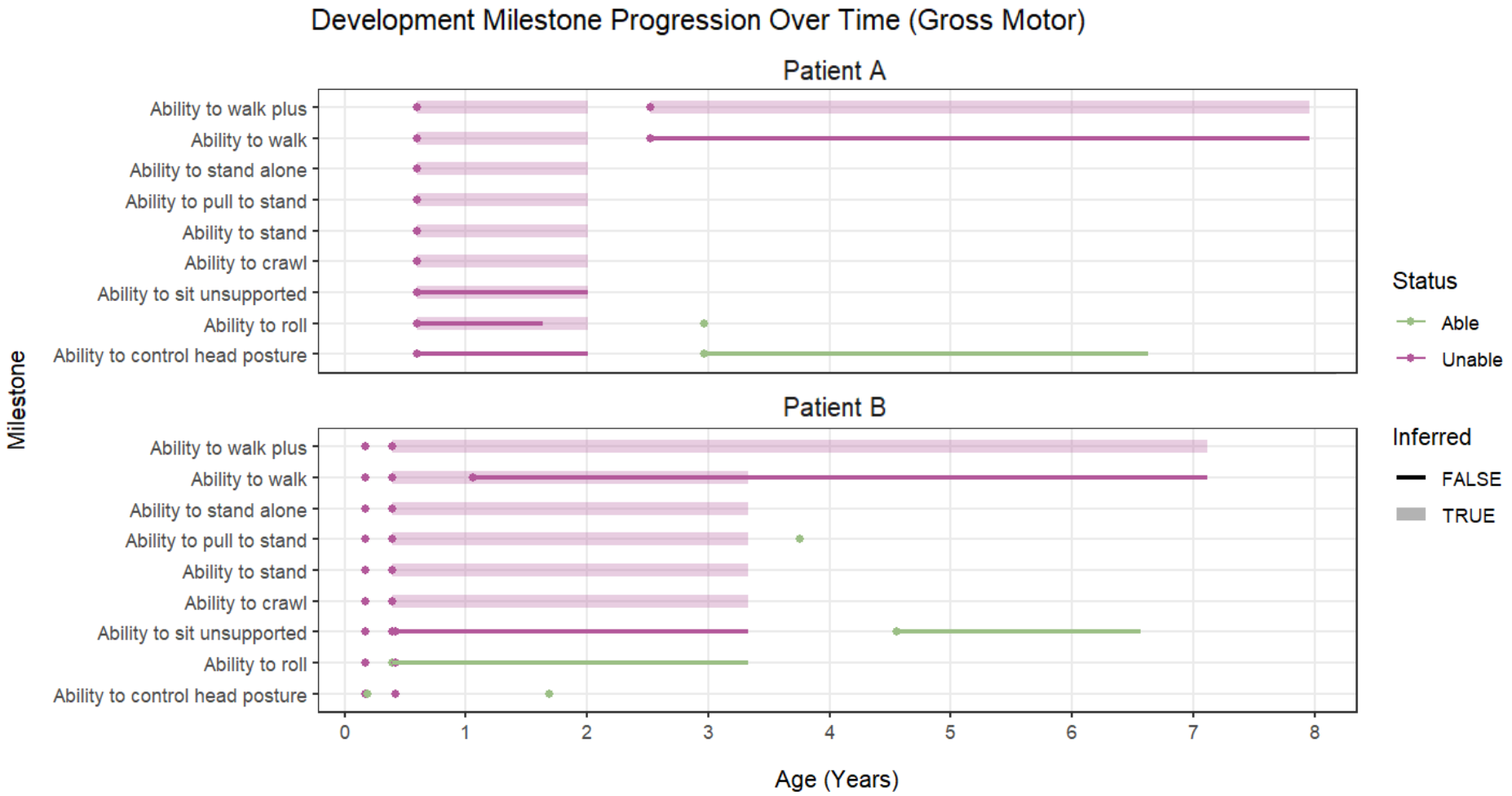
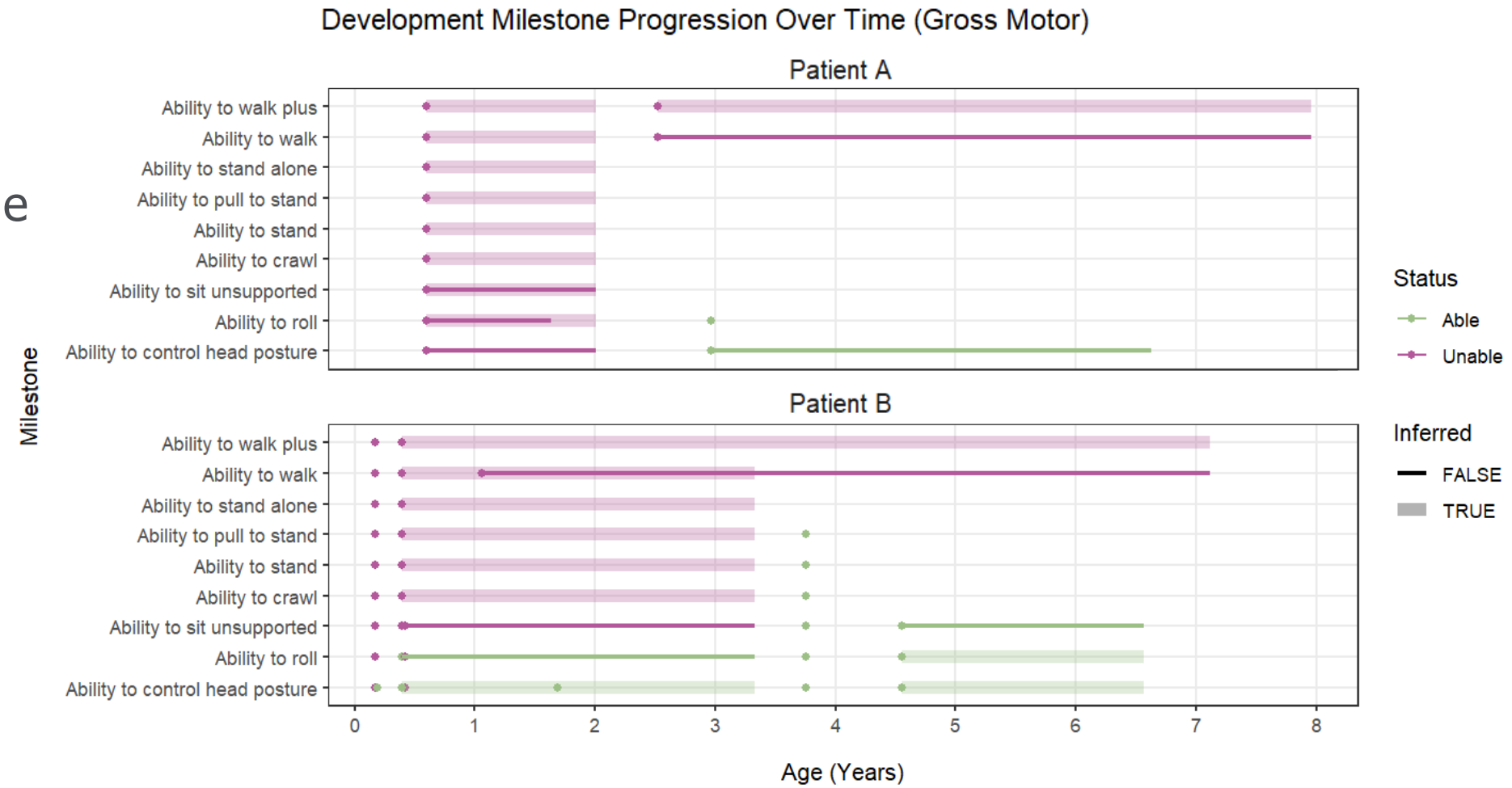


Developmental Milestones

Getting a more complete picture

1. Add groupings
2. Add ordering
3. No status change unless specified



Developmental Milestones

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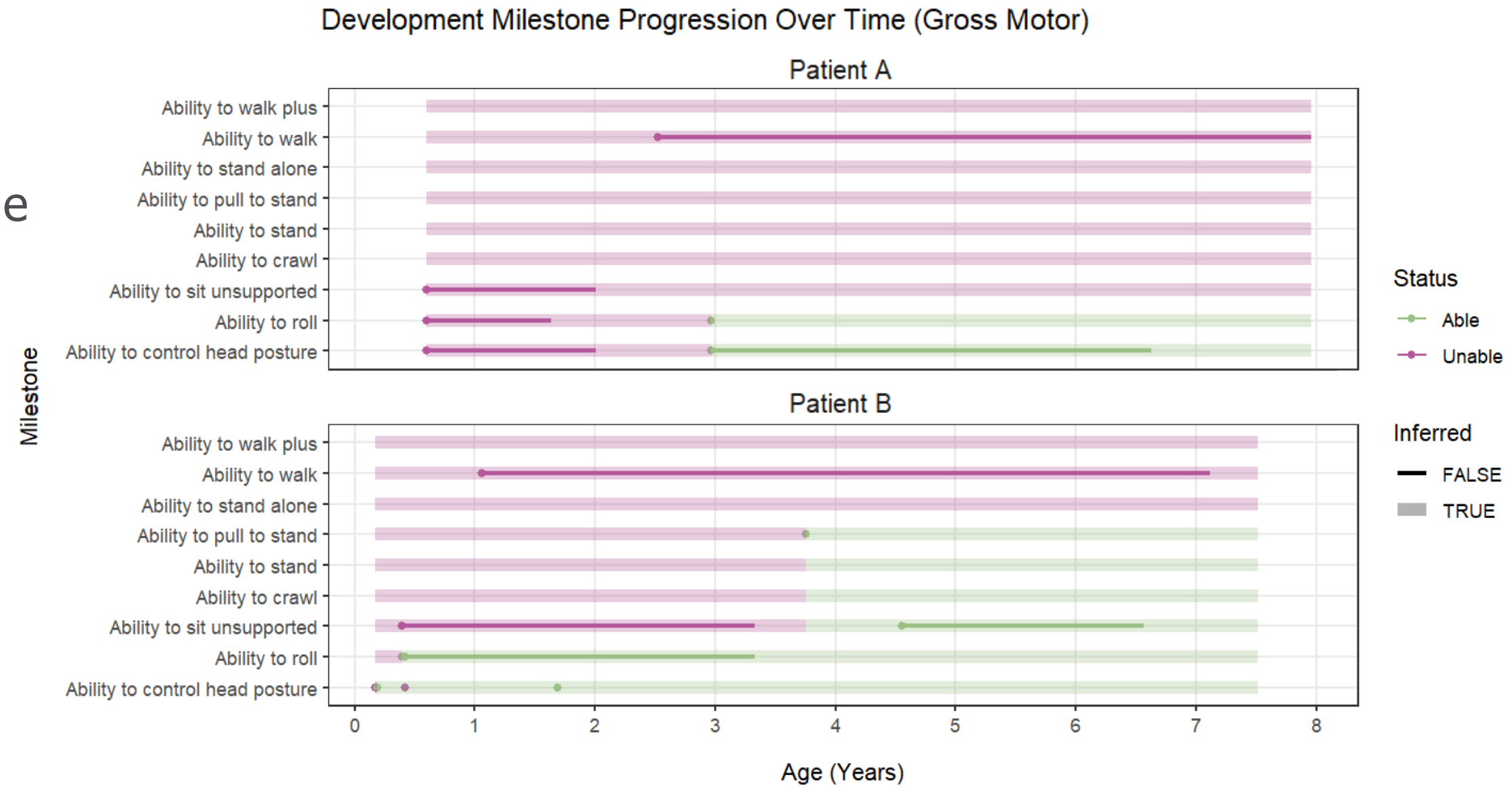
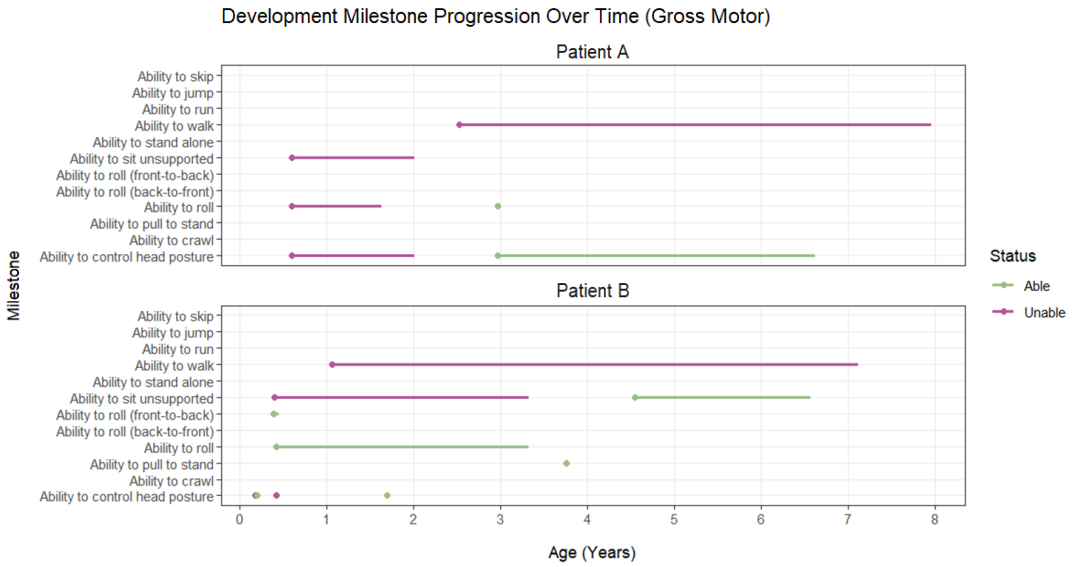
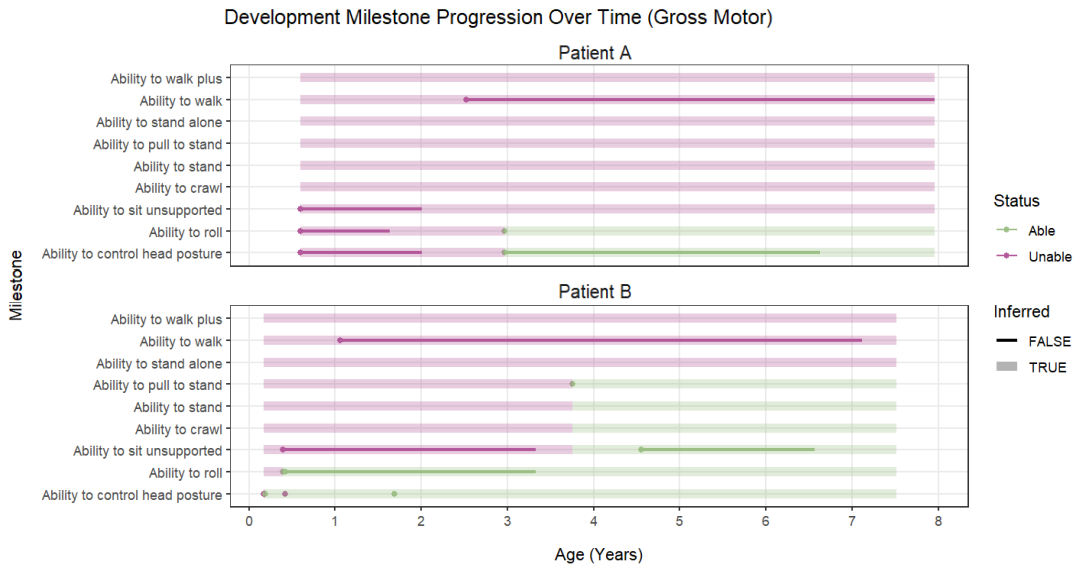


Developmental Milestones

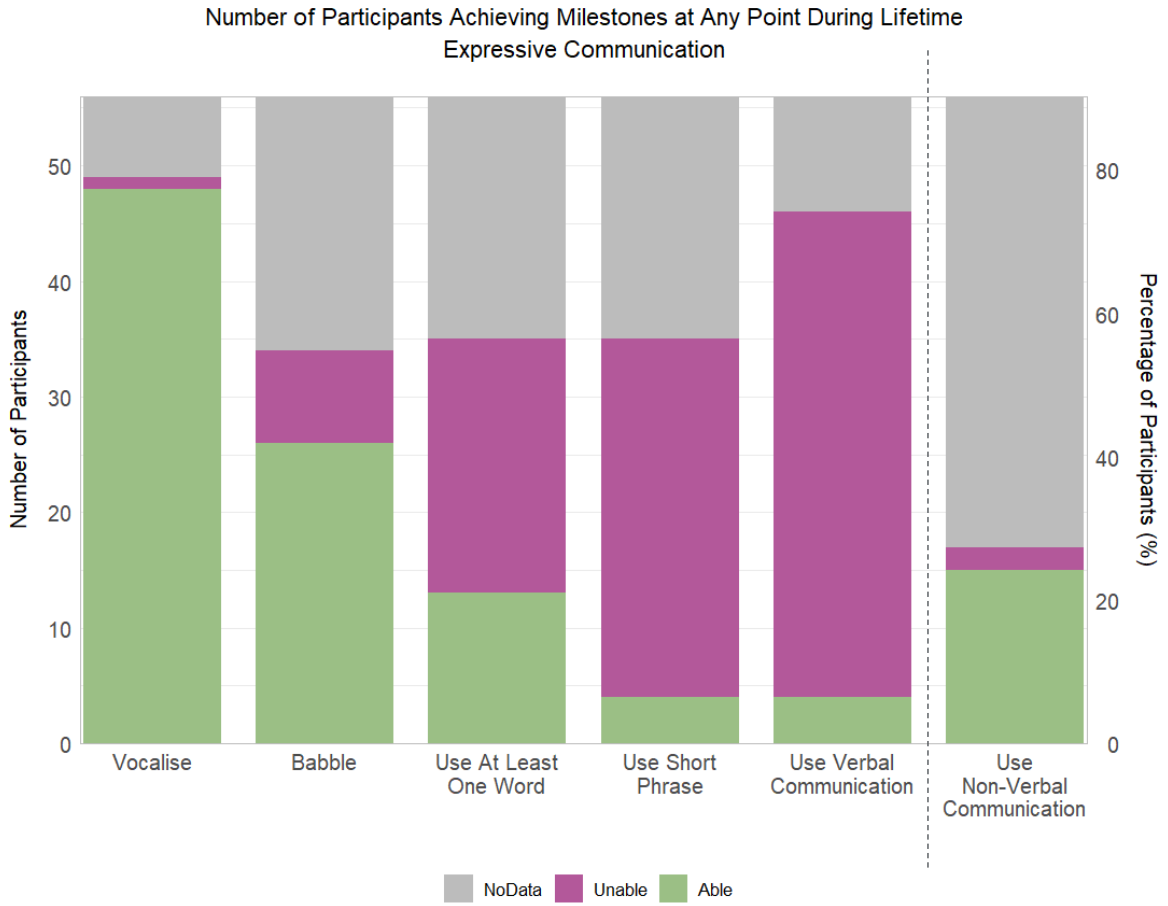
Getting a more complete picture





Developmental Milestones

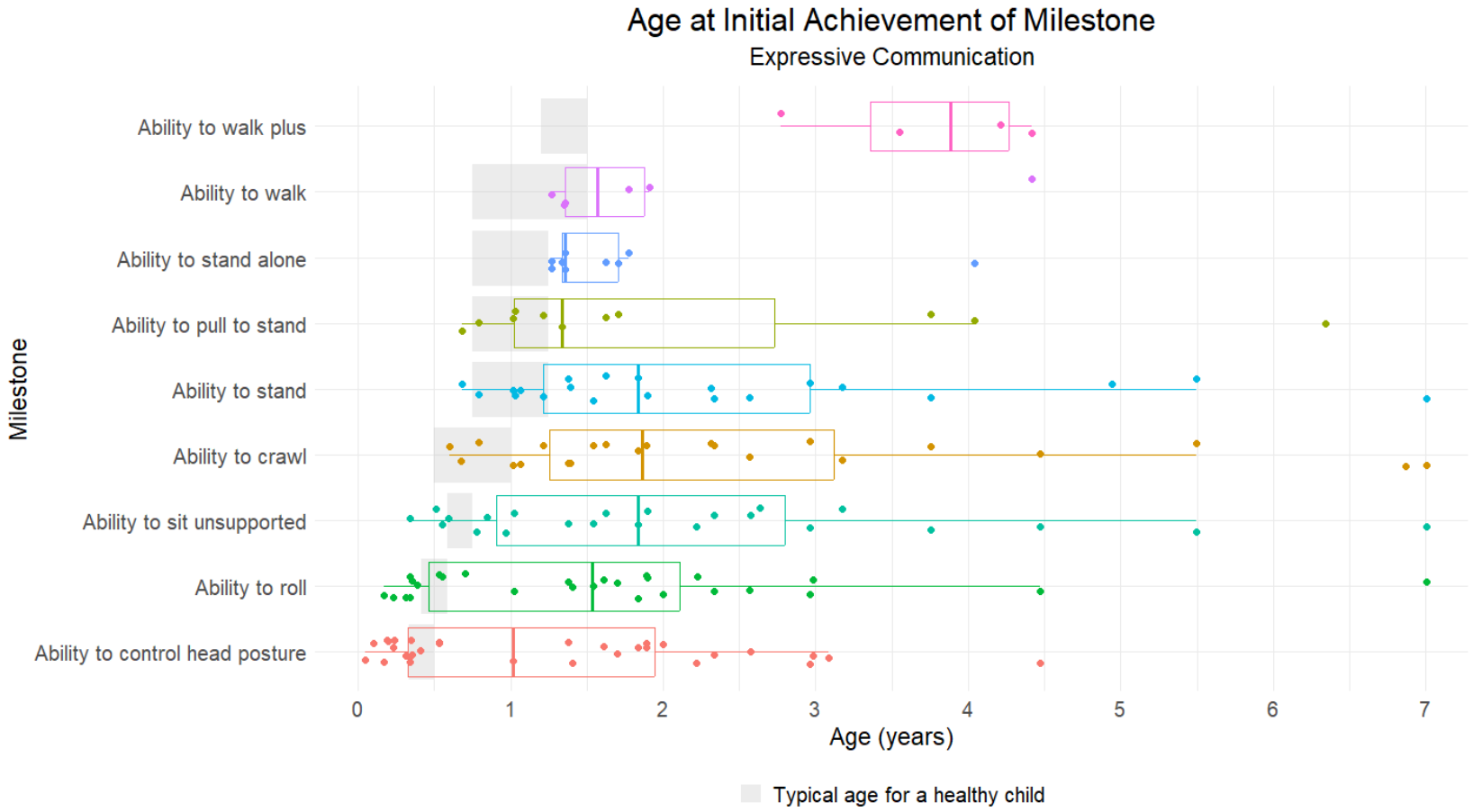
How many patients achieved each milestone?



Note: To be categorised as either Able or Unable, a patient must have some data for that milestone
Note: Non-verbal communication is considered separate from ordering

Developmental Milestones

What age are milestones achieved?

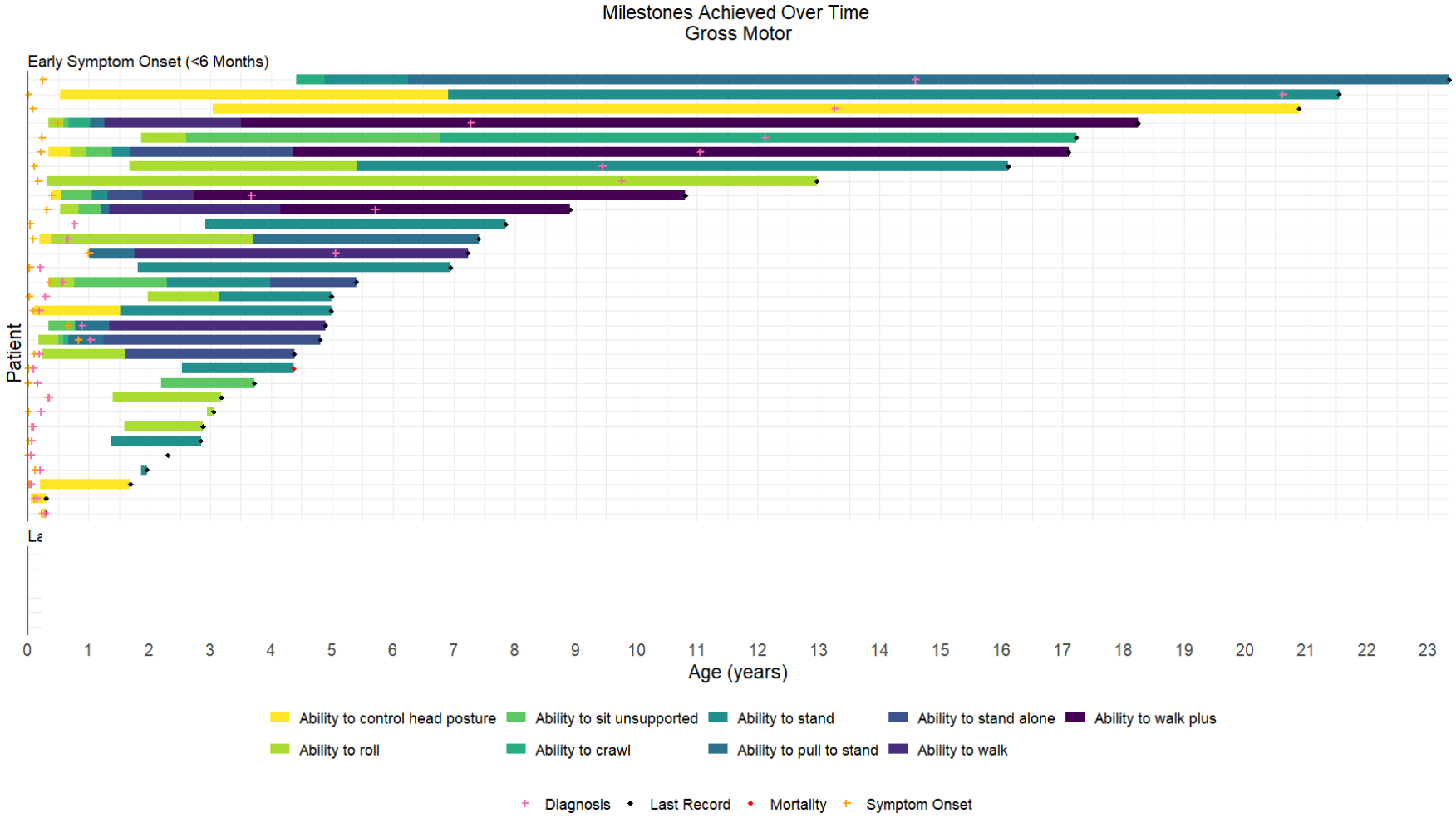


What do we learn?

- Early milestones have two groups: delay or no delay
- Later milestone achieved earlier: are patients achieving later milestones those without delay early on?

Developmental Milestones

What age are milestones achieved?



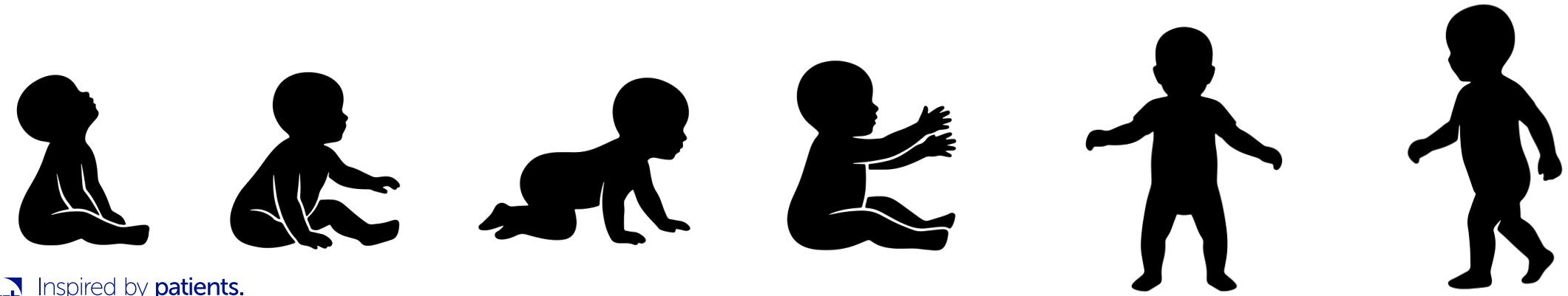
What do we learn?

- Patients achieving more difficult milestones have less delay early on
- Late onset patients less affected by developmental delay

Developmental Milestones

What did we learn?

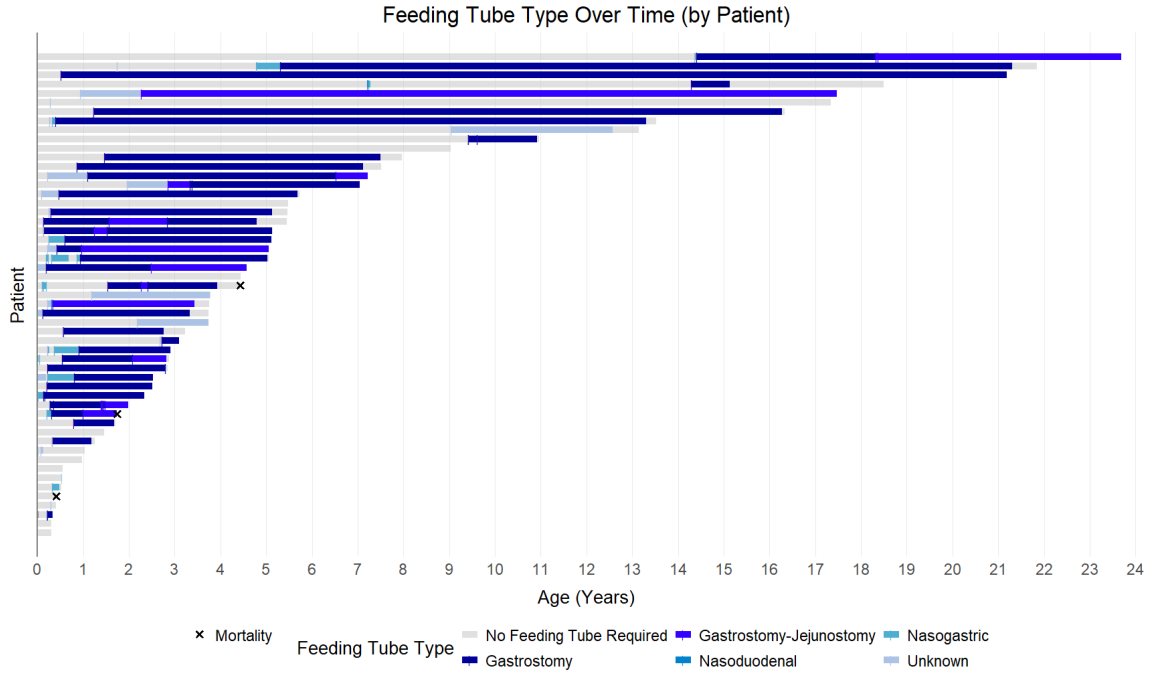
- Which milestones we can target to make a difference
    - In most cases! But we could clearly see where we weren't sure
- Patients who achieve later milestones mostly achieve them at a normal age
- Patients with late symptom onset achieve more milestones (at an earlier age)



Feeding Tube and Ventilator Usage

How many patients use a feeding tube?

83%

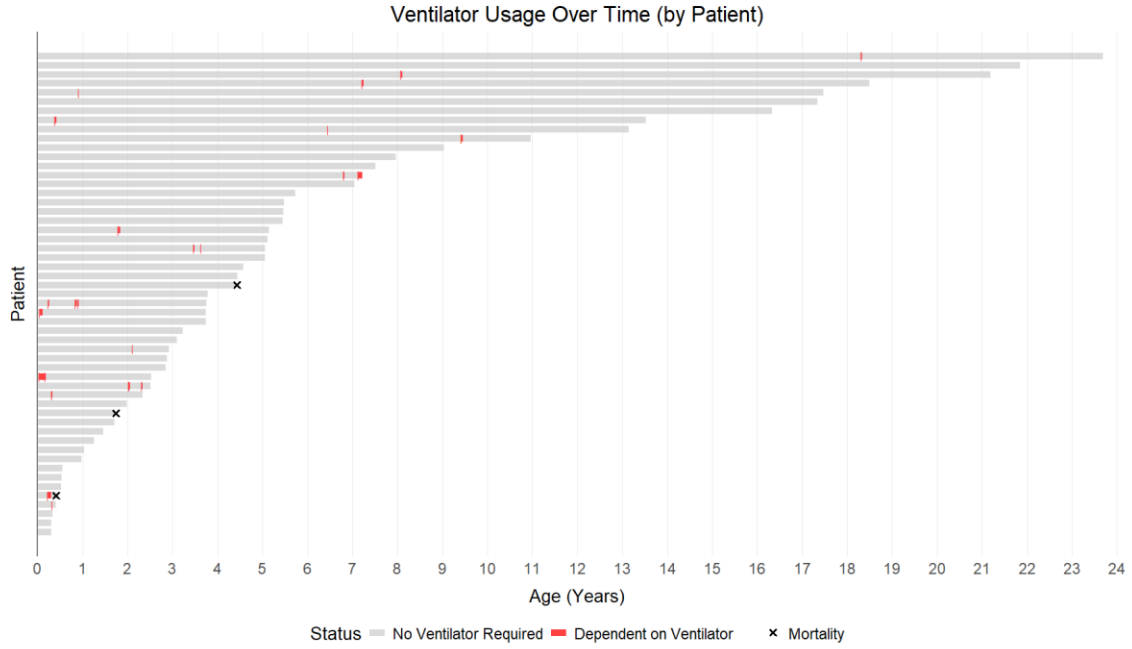


81% records > 10 days duration
72% records > 45 days duration

Low rate of recovery

How many patients use a ventilator?

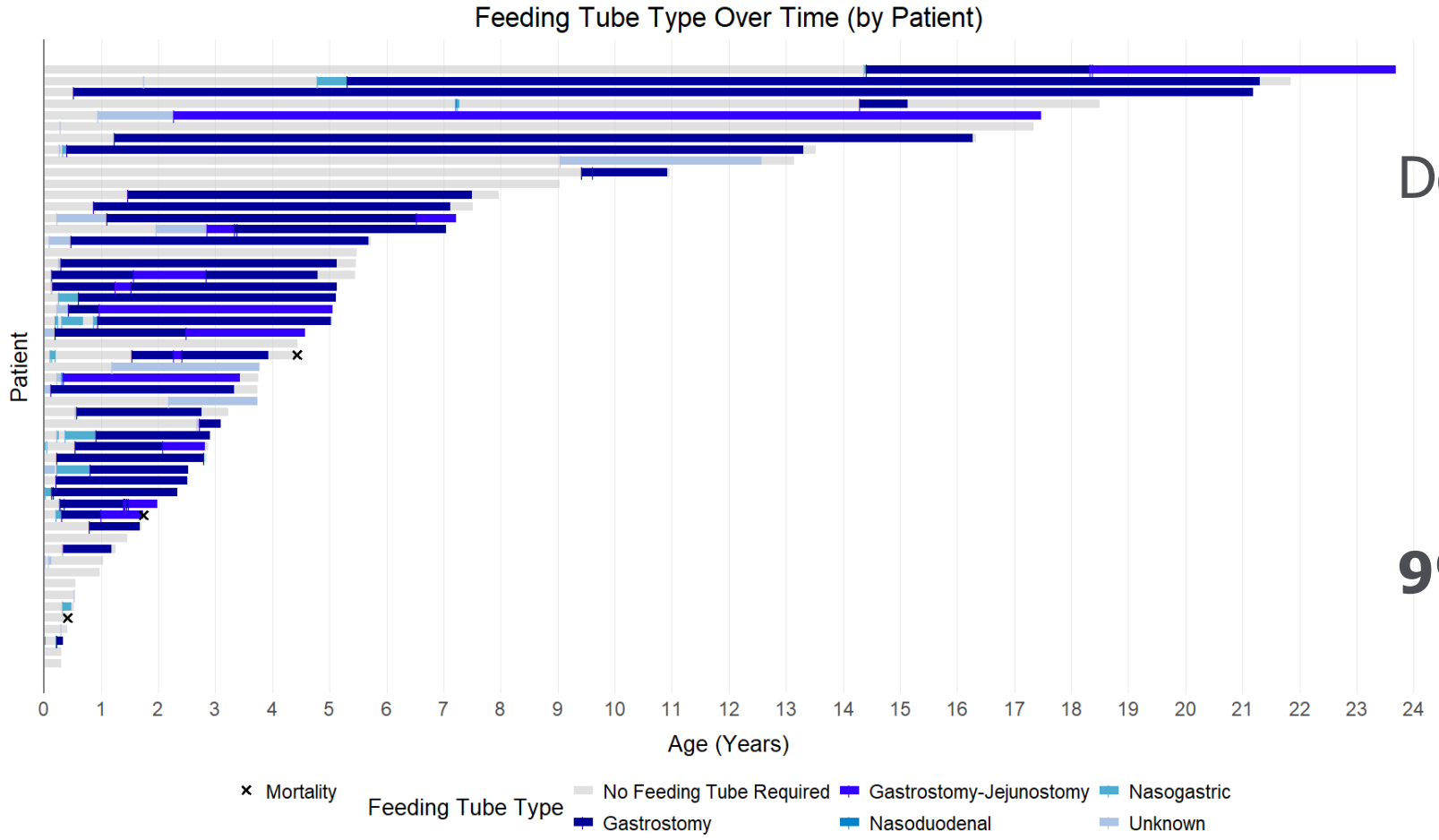
38%



17% records > 10 days duration
0% records > 45 days duration

Feeding Tube Usage

How many patients use a feeding tube?

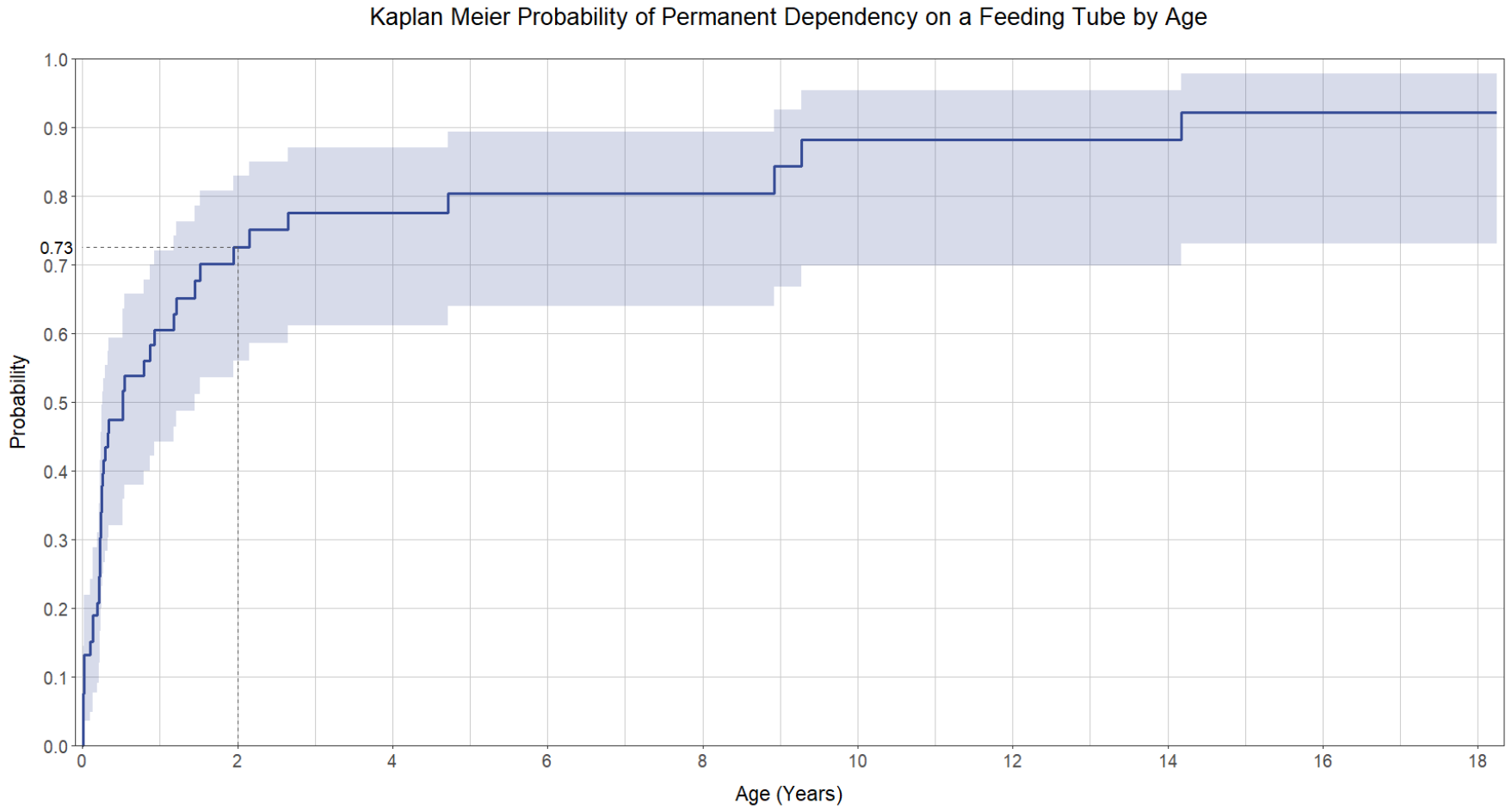


Define recovery =
at least one year without needing another feeding tube following removal of previous tube

9% recovery rate

Feeding Tube Usage

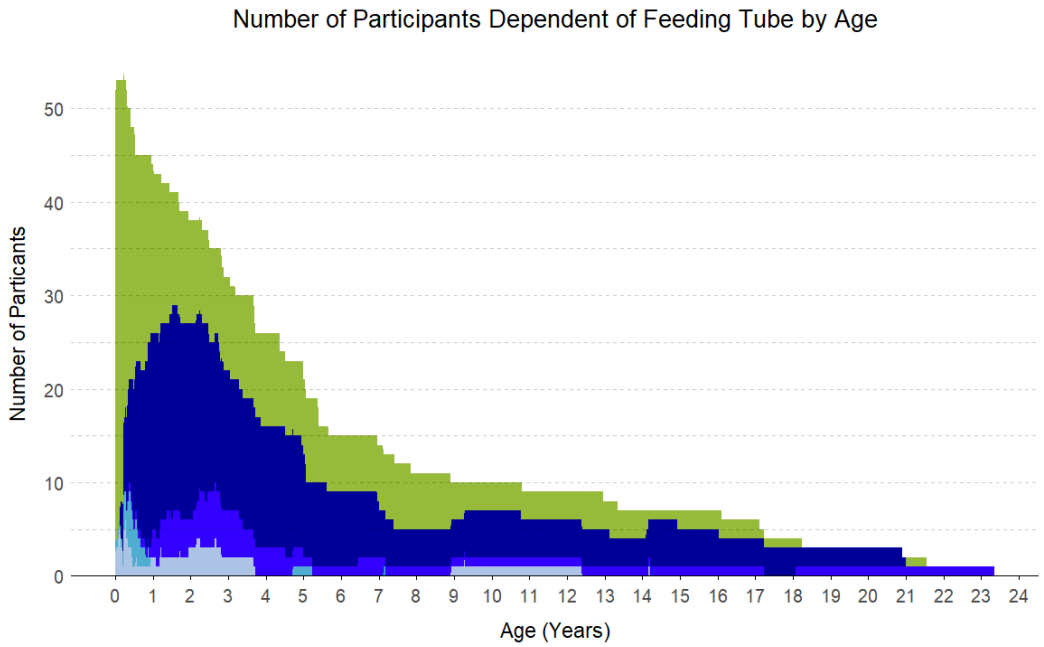
What age do patients become permanently dependent on a feeding tube?

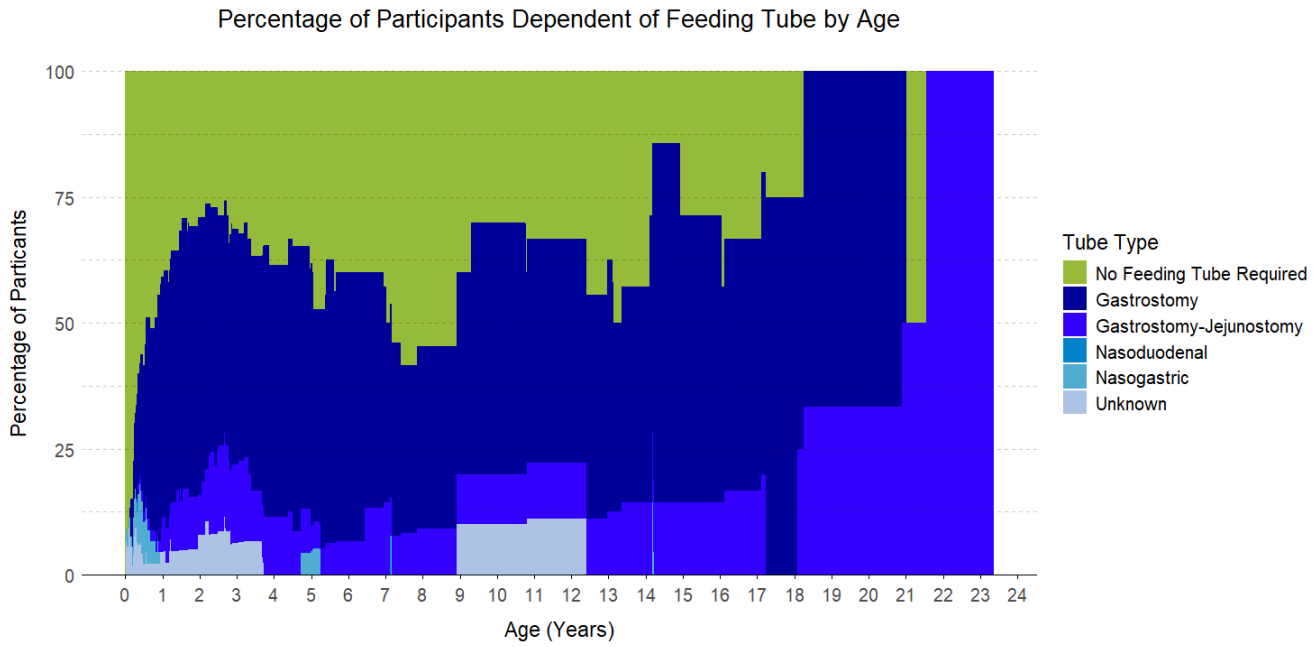


Note: Only dependencies without recovery were included in this plot
This is the complement of the Kaplan Meier plot ie 1 - prob(sruvival) where survival is no permanant dependency on a feeding tube

Feeding Tube and Ventilator Usage

What type of feeding tube do patients use?





Gastrostomy is most common type of tube

Feeding Tube and Ventilator Usage

What did we learn?

- Feeding tube usage is common and terminal, preventing it would have big impact on patients lives
    - 70% chance of being permanently dependent on a feeding tube by 2 years
- Most common type of feeding tube is gastrostomy
- Ventilator usage is typically short term, reducing it would be less impactful

Why use visualisations?

Without visualisations:

Little known about disease

Limited, messy and sparse data

→ Difficult to get information from the data

Using visualisations:

- We can make use of limited and messy data to better understand the disease
- Get a clearer direction of how we can make an impact
- Better design studies

Visualisations turn **limited, sparse, messy data** into **useful insights** - helping us **understand a disease** where data are difficult to obtain.

Thank you
Any questions?