Ongoing Clinical Trials by Phase and Therapeutic Approach

Alliance *for* Regenerative Medicine

Therapeutic Approach	Phase 1	Phase 2	Phase 3
Tissue Engineering	7	22	5
Gene Therapy	95	229	57
CBIO	413	349	21
Cell Therapy	319	584	119
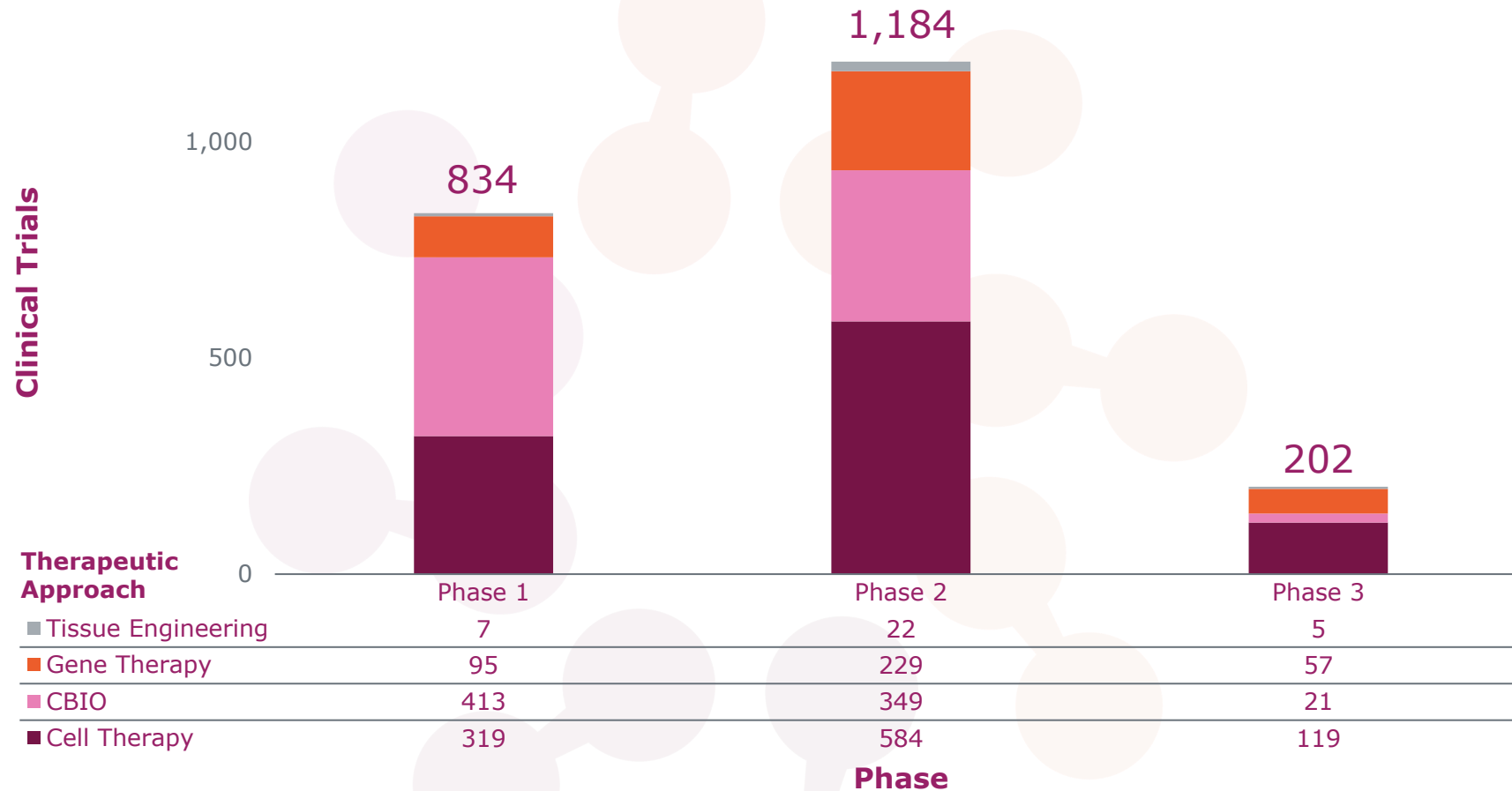
Phase

1. *Within-graph labels represent phase totals*

Ongoing Clinical Trials by Phase and Region

Region	Phase 1	Phase 2	Phase 3
Total	834	1,184	202
▪ Other Regions	48	56	35
▪ Europe	77	251	75
▪ Asia Pacific	337	431	80
▪ North America	339	526	99
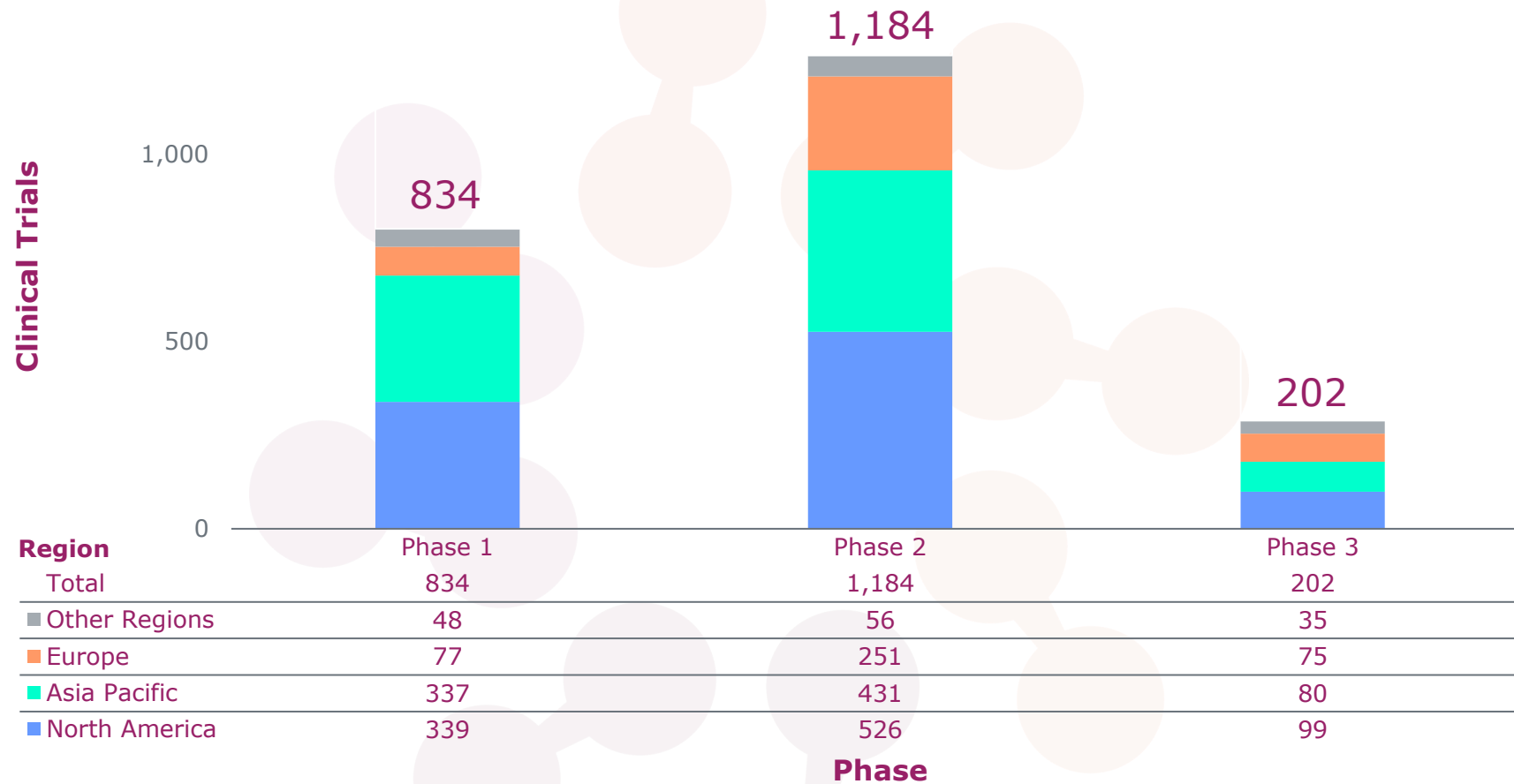
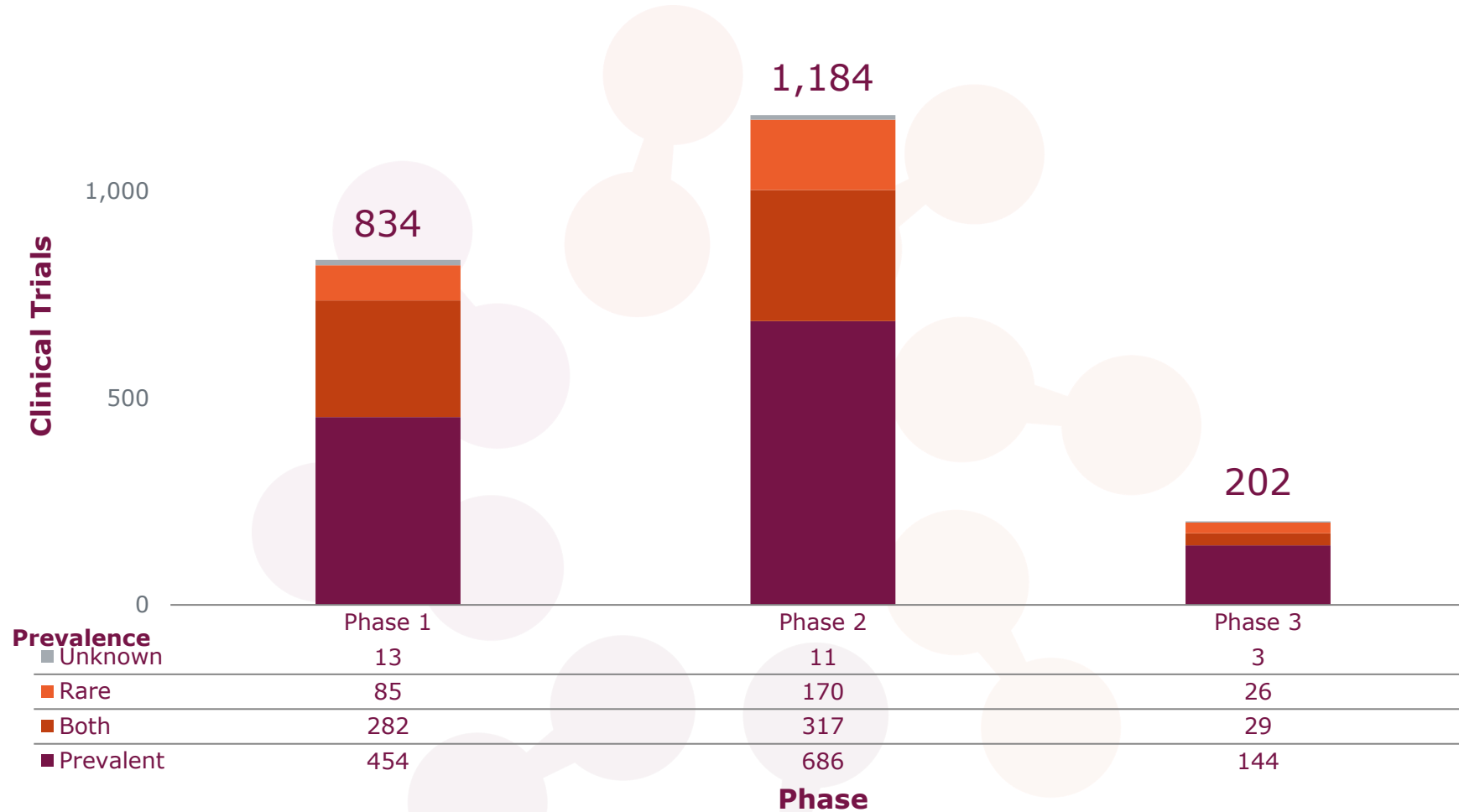1. Within-graph labels represent phase totals
2. Clinical trials take place in multiple regions; sum of regional totals will not be equivalent to phase totals

Ongoing Clinical Trials by Phase and Sponsor
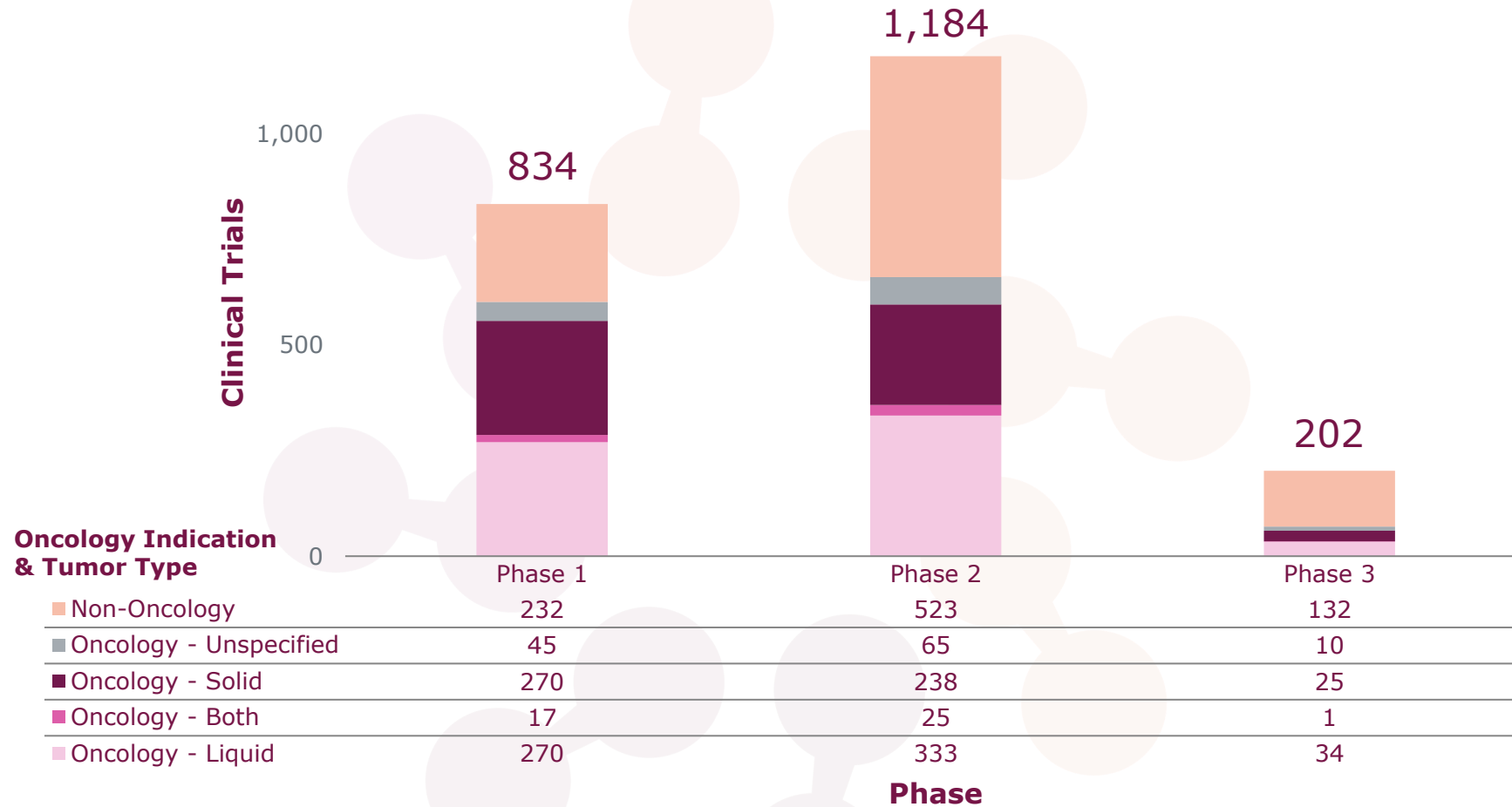

Sponsor	Phase 1	Phase 2	Phase 3
■ Nonindustry	435	641	74
■ Industry	399	543	128
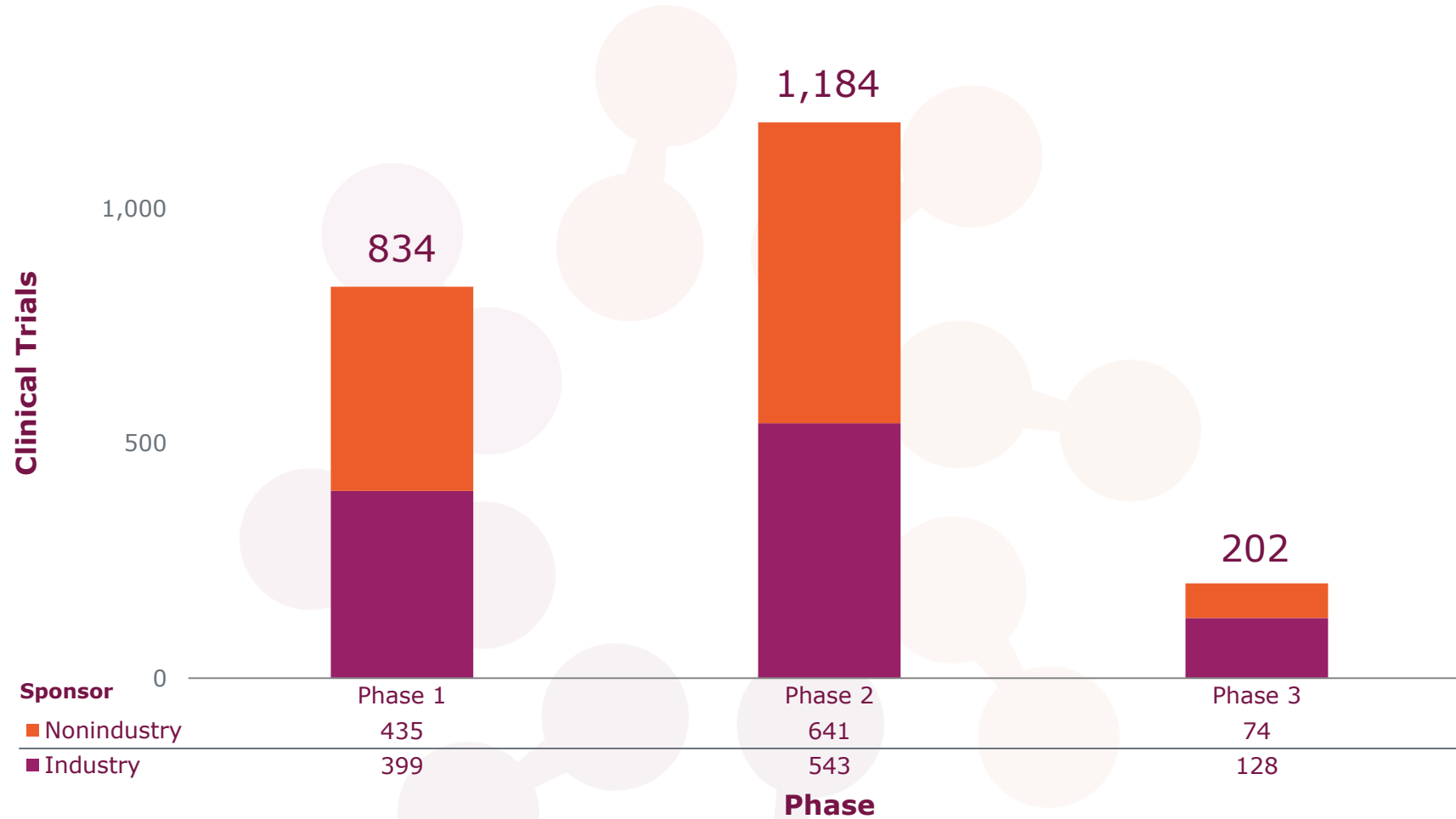
1. Within-graph labels represent phase totals
2. Non-industry includes academic and governement sponsors

Ongoing Clinical Trials by Phase and Prevalence

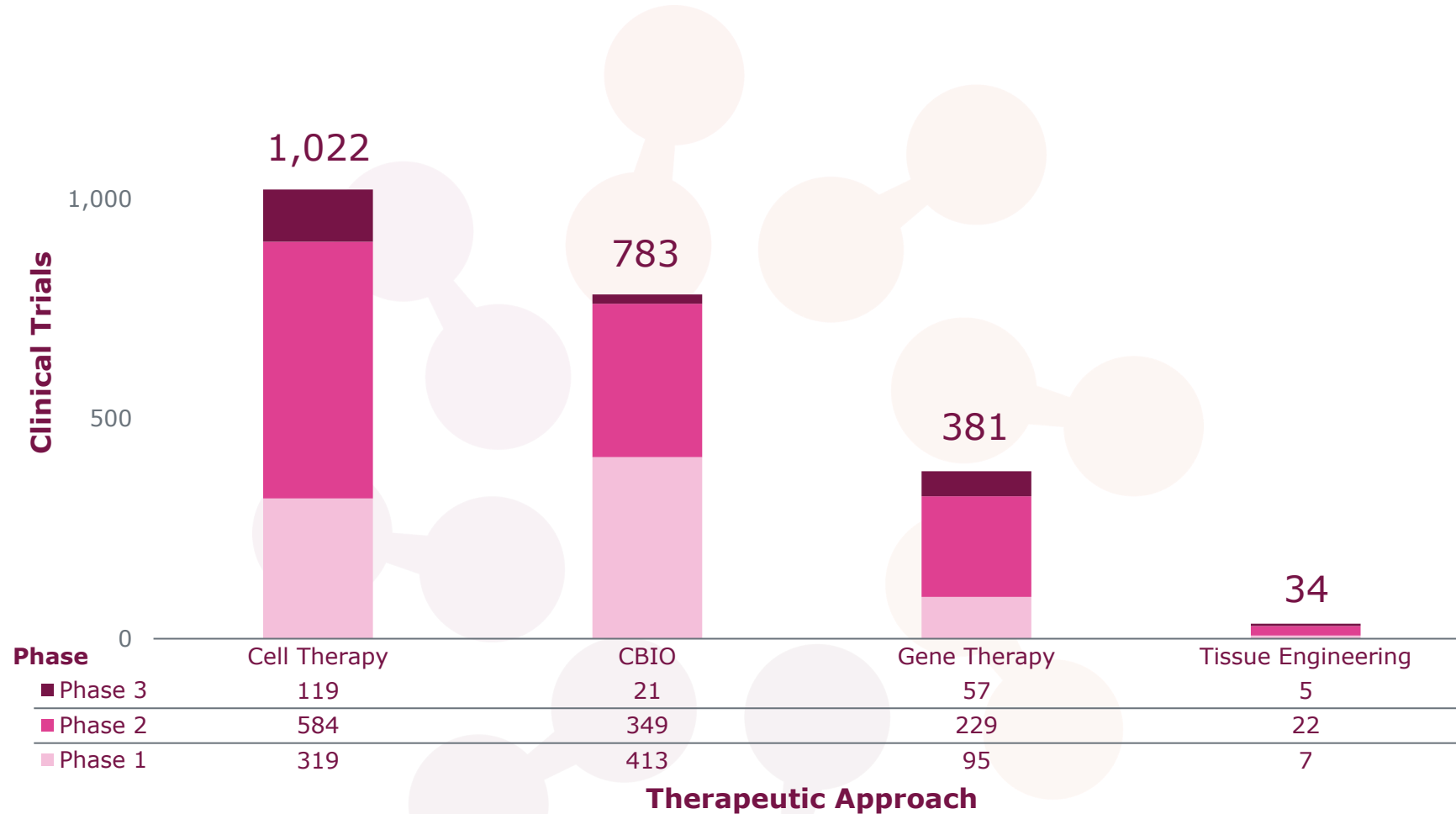| Prevalence	Phase 1	Phase 2	Phase 3
■ Unknown	13	11	3
■ Rare	85	170	26
■ Both	282	317	29
■ Prevalent	454	686	144

Clinical Trials (y-axis)

Phase (x-axis)

1. *Within-graph labels represent phase totals*

Ongoing Clinical Trials by Phase and Oncology Indication & Tumor Type

Oncology Indication & Tumor Type	Phase 1	Phase 2	Phase 3
Non-Oncology	232	523	132
Oncology - Unspecified	45	65	10
Oncology - Solid	270	238	25
Oncology - Both	17	25	1
Oncology - Liquid	270	333	34

1. Within-graph labels represent phase totals

Ongoing Clinical Trials by Therapeutic Approach and Phase

Clinical Trials

Phase	Cell Therapy	CBIO	Gene Therapy	Tissue Engineering
Phase 3	119	21	57	5
Phase 2	584	349	229	22
Phase 1	319	413	95	7

Therapeutic Approach

1. Within-graph labels represent therapeutic approach totals

Ongoing Clinical Trials by Therapeutic Approach and Region

Region Total	Cell Therapy 1,022	CBIO 783	Gene Therapy 381	Tissue Engineering 34
Other Regions	98	13	27	1
Europe	161	114	119	9
Asia Pacific	340	399	97	12
North America	406	305	242	11

Therapeutic Approach
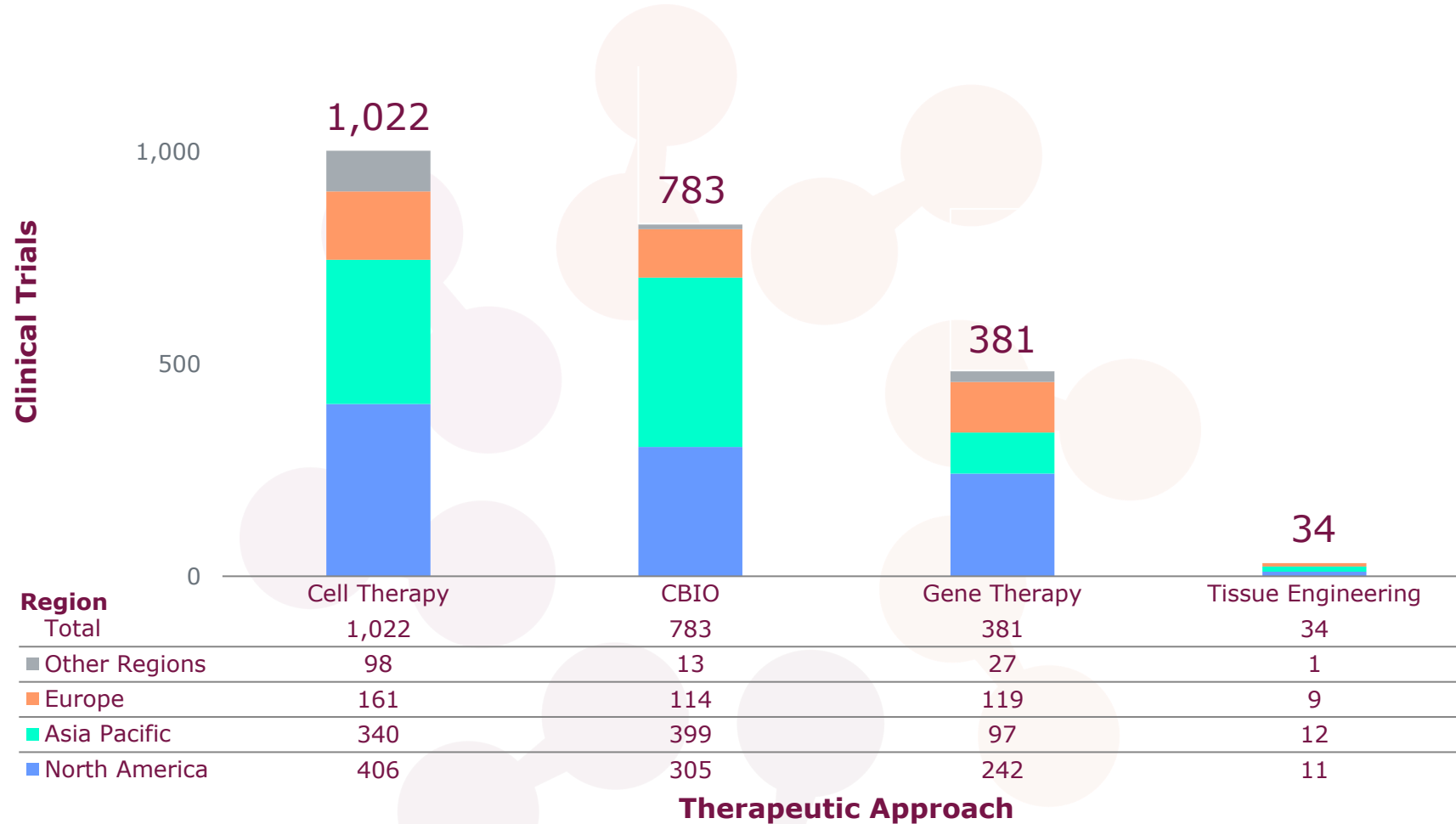
1. Within-graph labels represent therapeutic approach totals
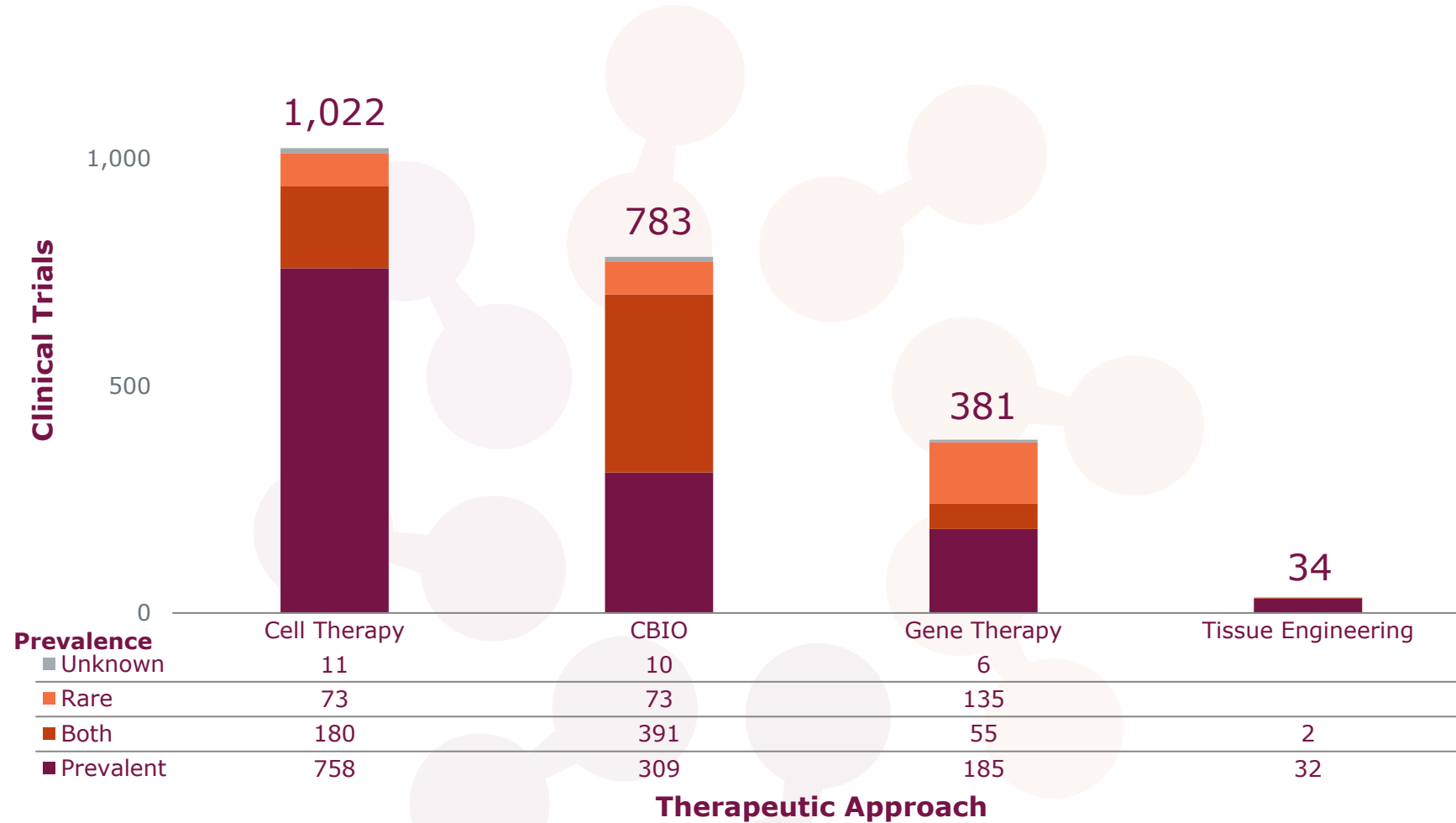2. Clinical trials take place in multiple regions; sum of regional totals will not be equivalent to therapeutic approach totals

Ongoing Clinical Trials by Therapeutic Approach and Sponsor

Alliance *for* **Regenerative Medicine**

Clinical Trials

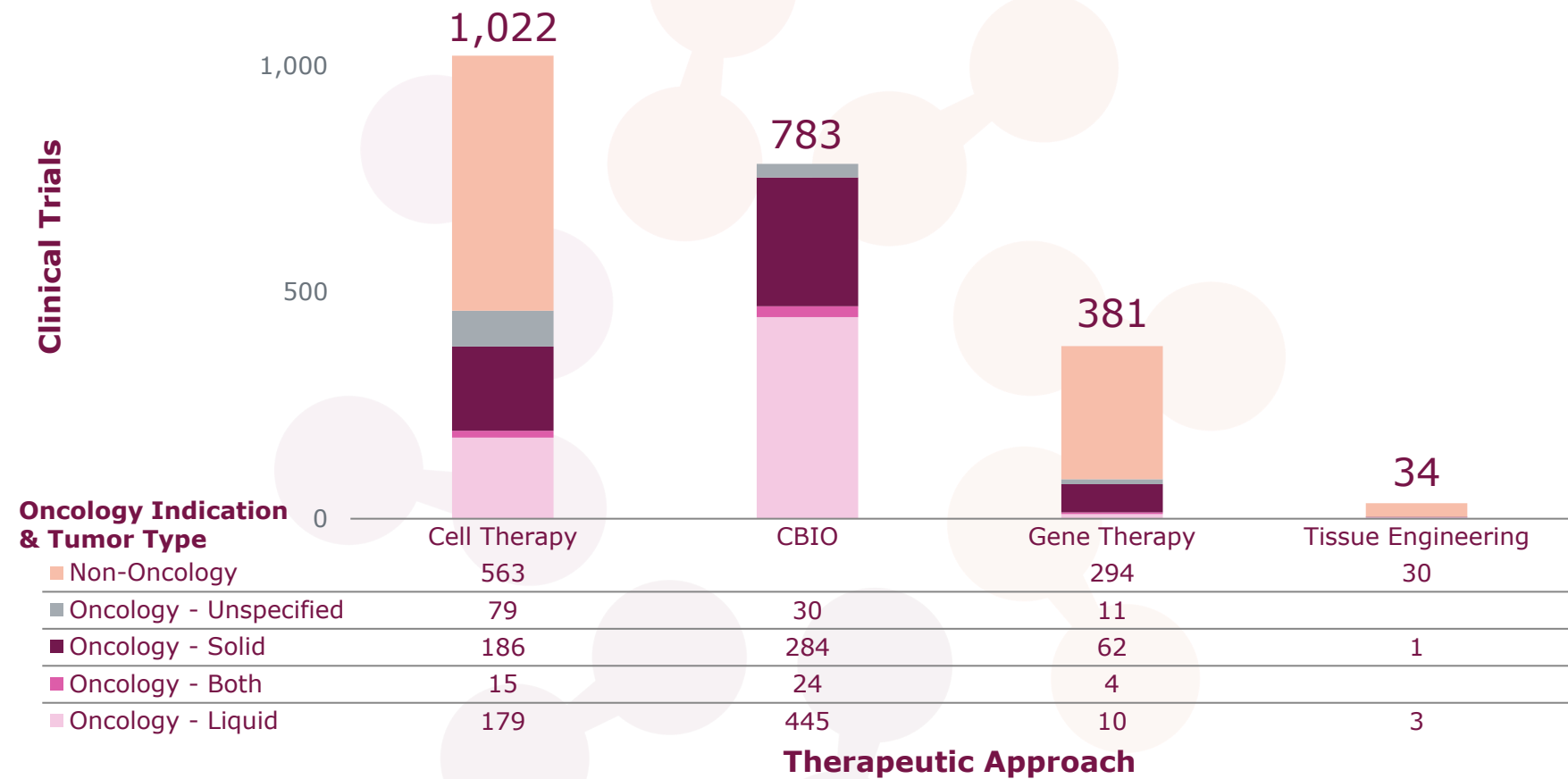| Sponsor	Cell Therapy	CBIO	Gene Therapy	Tissue Engineering
Nonindustry	645	370	121	14
Industry	377	413	260	20

Therapeutic Approach
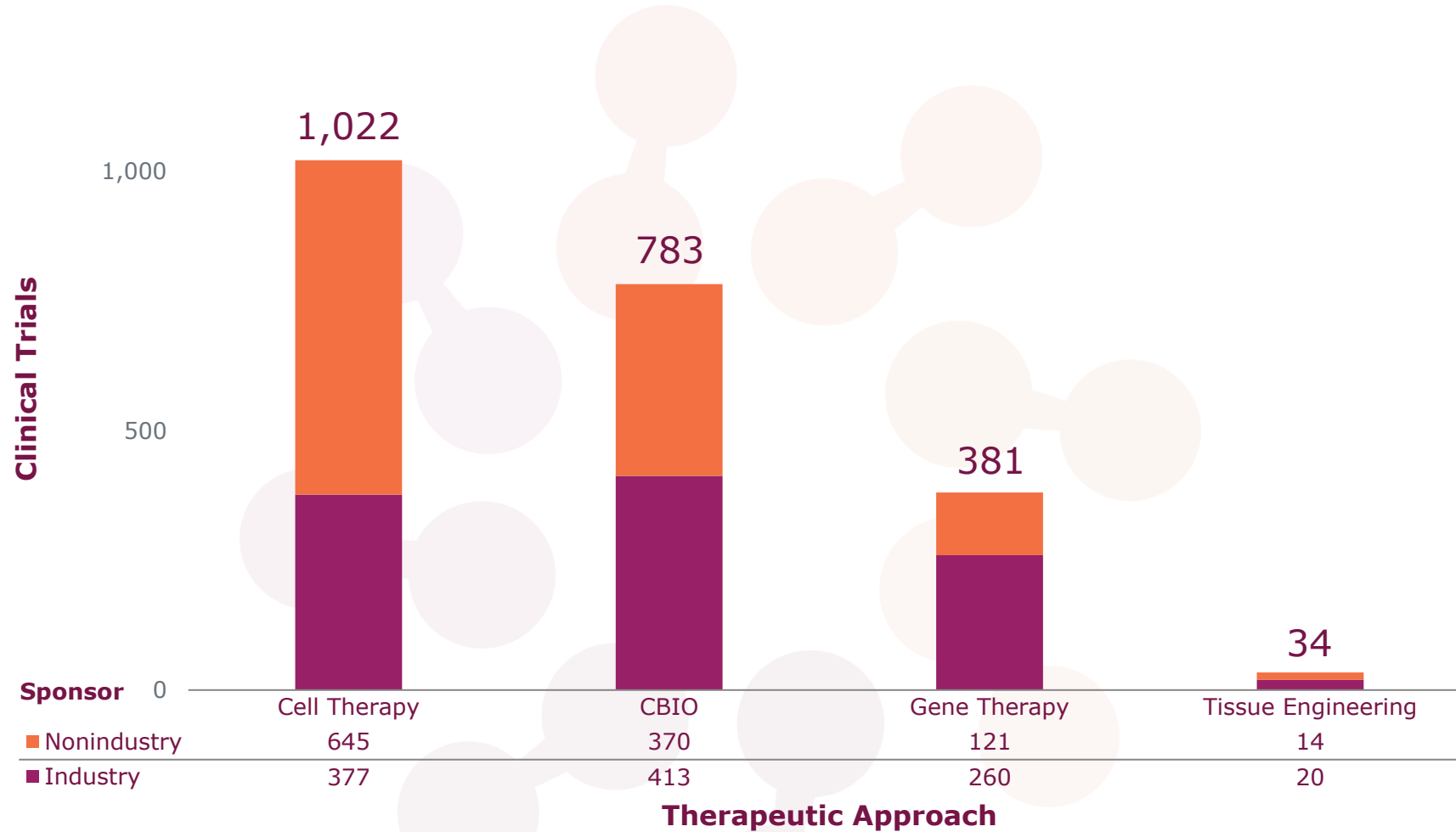
1. Within-graph labels represent therapeutic approach totals
2. Non-industry includes academic and governement sponsors

Ongoing Clinical Trials by Therapeutic Approach and Prevalence

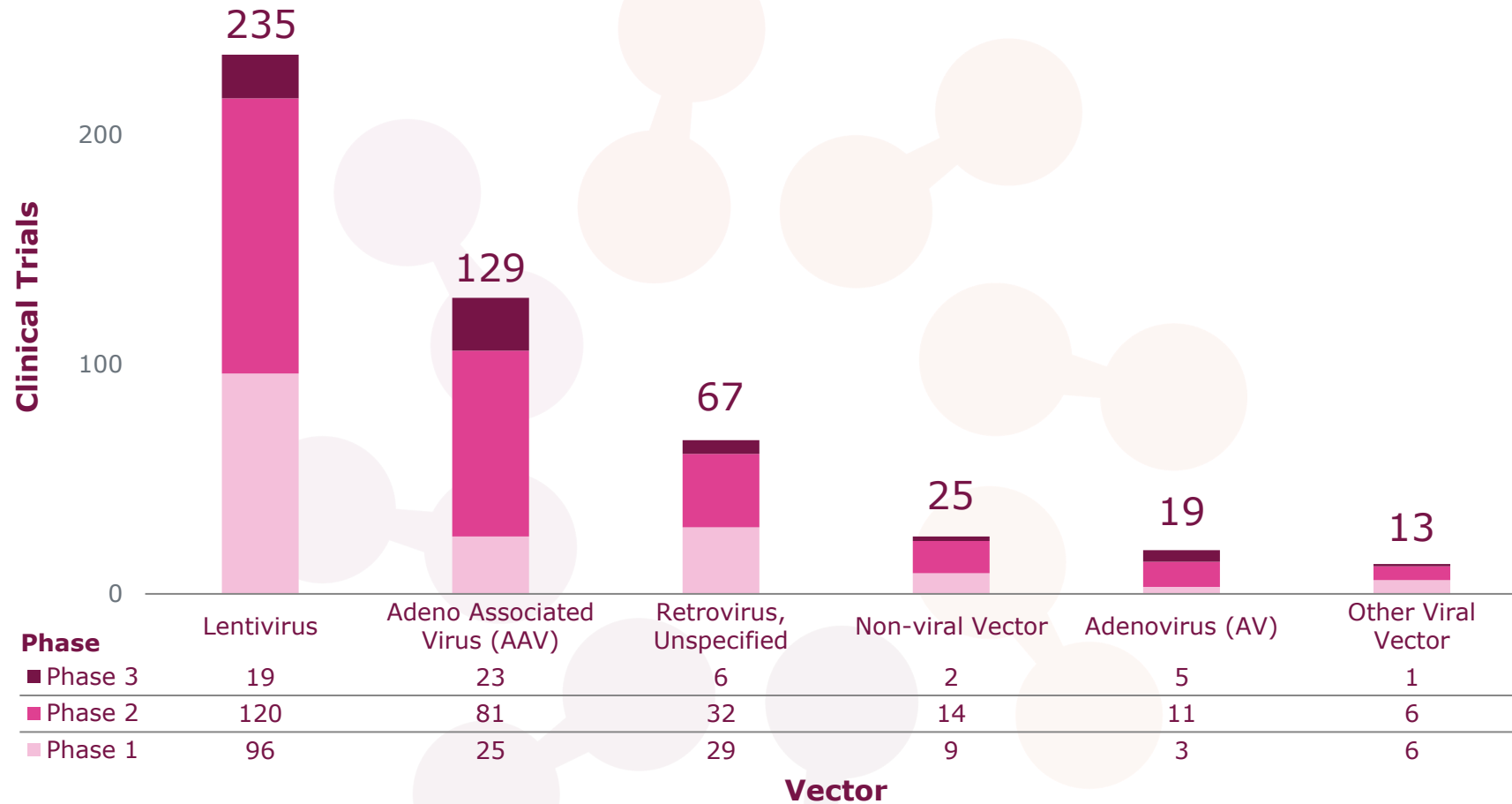| Prevalence	Cell Therapy	CBIO	Gene Therapy	Tissue Engineering
■ Unknown	11	10	6	
■ Rare	73	73	135	
■ Both	180	391	55	2
■ Prevalent	758	309	185	32

Therapeutic Approach

1. Within-graph labels represent therapeutic approach totals

Ongoing Clinical Trials by Therapeutic Approach and Oncology Indication & Tumor Type

Oncology Indication & Tumor Type	Cell Therapy	CBIO	Gene Therapy	Tissue Engineering
■ Non-Oncology	563		294	30
■ Oncology - Unspecified	79	30	11	
■ Oncology - Solid	186	284	62	1
■ Oncology - Both	15	24	4	
■ Oncology - Liquid	179	445	10	3

Therapeutic Approach

1. Within-graph labels represent therapeutic approach totals

Ongoing Clinical Trials by Vector and Phase

Alliance for Regenerative Medicine

Clinical Trials (y-axis)

Vector (x-axis)

Bar totals: Lentivirus 235, Adeno Associated Virus (AAV) 129, Retrovirus, Unspecified 67, Non-viral Vector 25, Adenovirus (AV) 19, Other Viral Vector 13

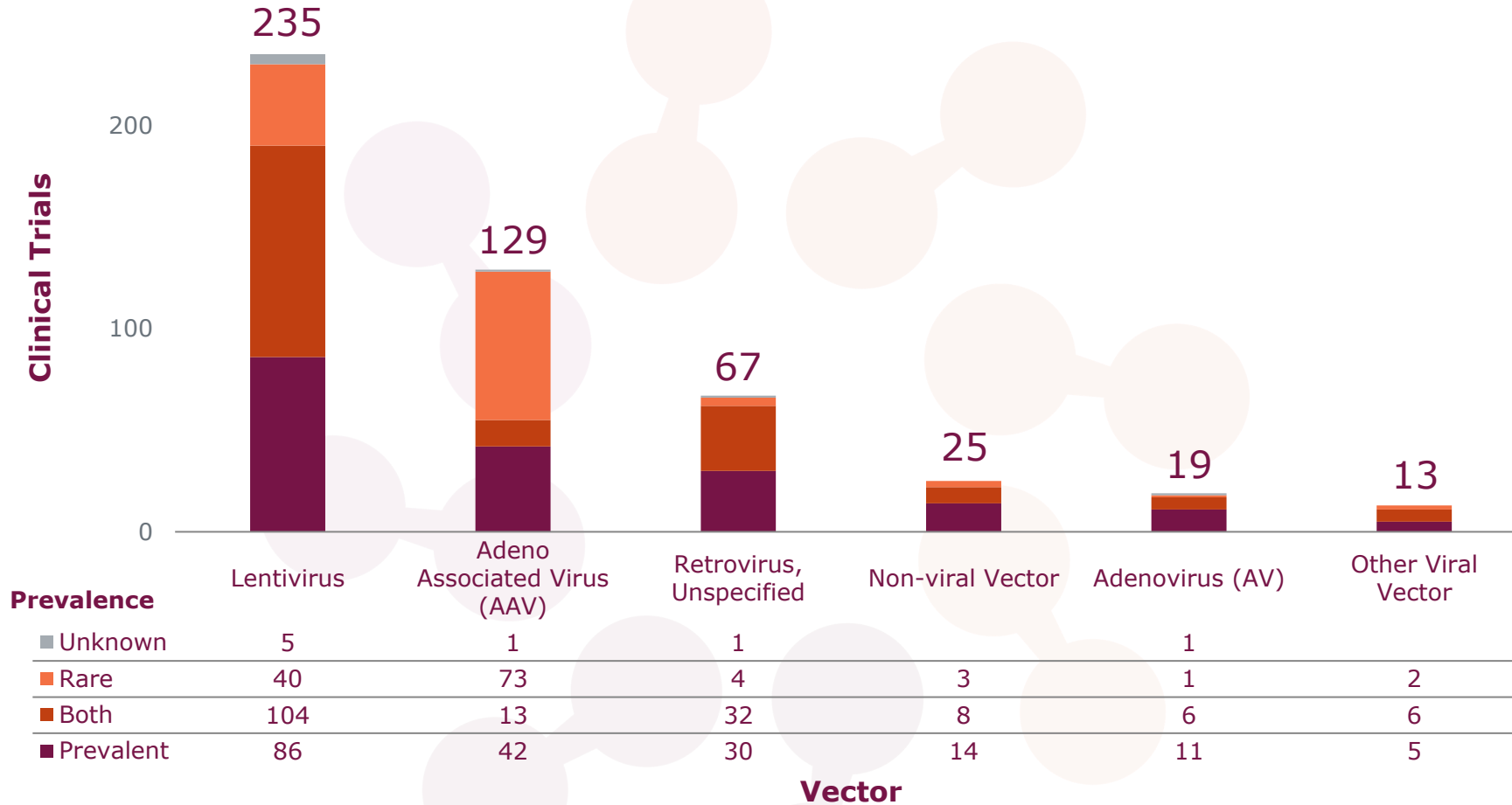| Phase	Lentivirus	Adeno Associated Virus (AAV)	Retrovirus, Unspecified	Non-viral Vector	Adenovirus (AV)	Other Viral Vector
Phase 3	19	23	6	2	5	1
Phase 2	120	81	32	14	11	6
Phase 1	96	25	29	9	3	6

1. Within-graph labels represent vector totals

Ongoing Clinical Trials by Vector and Therapeutic Approach

Therapeutic Approach	Lentivirus	Adeno Associated Virus (AAV)	Retrovirus, Unspecified	Non-viral Vector	Adenovirus (AV)	Other Viral Vector
Gene Therapy	61	121	6	18	18	4
CBIO	174	8	61	7	1	9
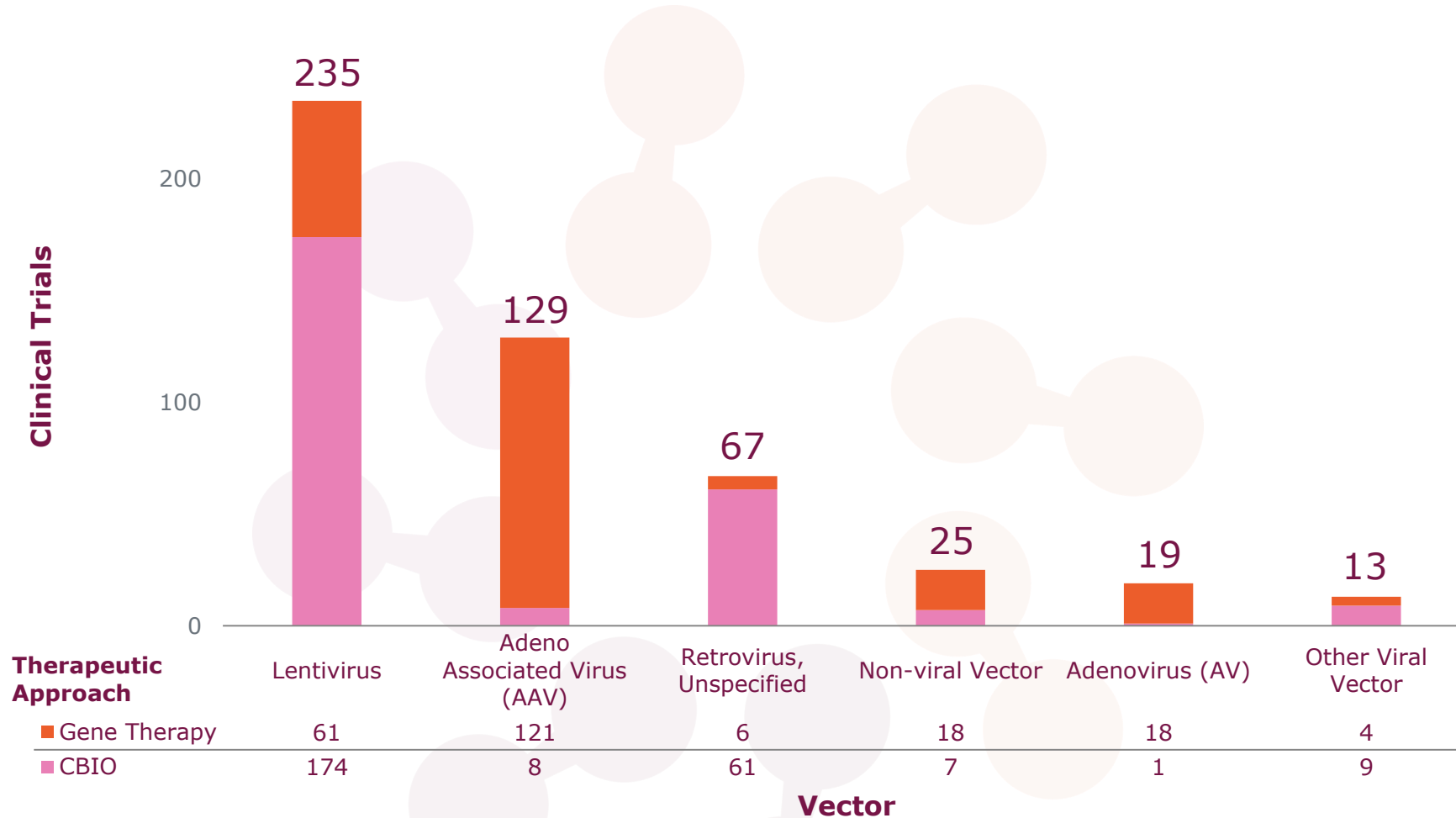
1. Within-graph labels represent vector totals

Ongoing Clinical Trials by Vector and Prevalence

Prevalence	Lentivirus	Adeno Associated Virus (AAV)	Retrovirus, Unspecified	Non-viral Vector	Adenovirus (AV)	Other Viral Vector
■ Unknown	5	1	1		1	
■ Rare	40	73	4	3	1	2
■ Both	104	13	32	8	6	6
■ Prevalent	86	42	30	14	11	5

Vector

1. Within-graph labels represent vector totals

Ongoing Clinical Trials by Vector and Sponsor

Clinical Trials

235

129

67

25

19

13

200

100

0

Vector	Lentivirus	Adeno Associated Virus (AAV)	Retrovirus, Unspecified	Non-viral Vector	Adenovirus (AV)	Other Viral Vector
Sponsor						
Nonindustry	106	19	36	7	5	3
Industry	129	110	31	18	14	10
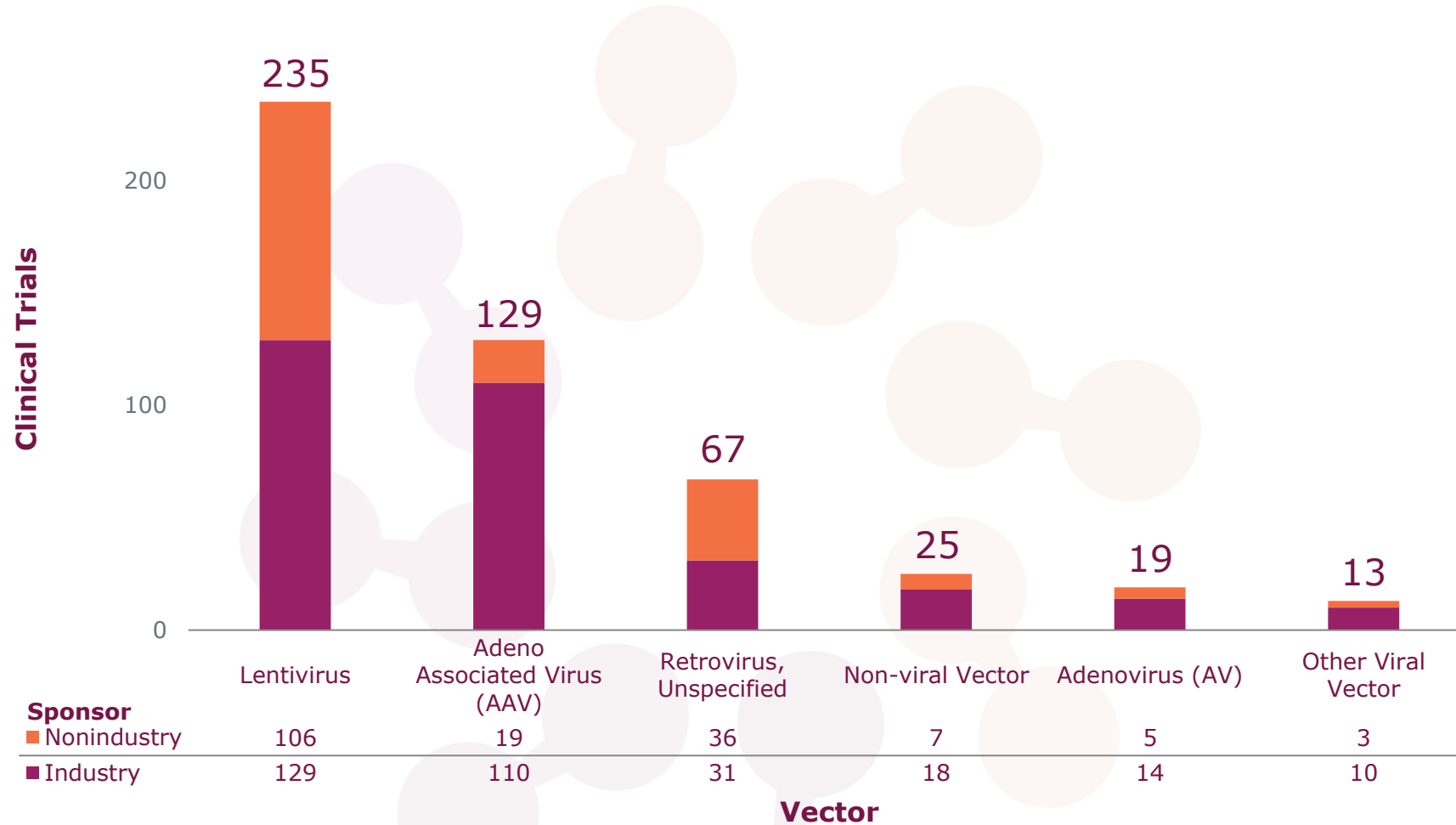
Vector

1. Within-graph labels represent vector totals
2. Non-industry includes academic and governement sponsors

Methodology Notes for Clinical Trial Data

Methodology Notes

1. Clinical trial data represent a snapshot in time; this snapshot was taken in December 2022.

2. Clinical trials include trials in phases 1-3, active at any point in the calendar year; phase 2 may include phase 1/2 trials, phase 3 may include phase 2/3 trials.

3. Clinical trial regions are based on known site locations, which may change over time.

4. Clinical trials may have multiple site locations which may reside in a single region or span across multiple regions; the sum of clinical trials across regions will not be equivalent to the total number of trials.

5. Nonindustry trials include academic and government sponsors, as well as hospitals, medical centers, and other facilities funded by not-for-profits, foundations, etc.

6. Prevalence classifications are based on GlobalData indentification of trial indications; many trials have multiple indications.

7. GlobalData's clasification of 'rare' indications or populations is based on US FDA and HIG Genetic and Rare Diseases Information Center (GARD) definitions (affecting <200,000 people in the US); 'rare' may not reflect different regional prevalences.