Gender Aspects in the Nordic ATMP Space 2023
ATMP2030



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Gender Equality in the Nordic ATMP Space

2023

### Introduction

- Gender equality has been discussed globally in recent years, and even though the gender gaps in education and workforce seem to have narrowed, there are limited data about the gender gap in the Nordic countries, and more specifically about the ATMP space.
- The objective of the "Gender Aspects in the Nordic ATMP Space" report requested by Vinnova is to investigate gender aspects parameters in the ATMP space in the Nordics, in comparison with other geographies. The selected parameters investigated were chosen in collaboration with RISE and do not reflect all aspects of equality in ATMP.
- Data from the 2023 Global Gender Gap Index, including the four subindexes "Economic participation and opportunity", "Educational attainment", "Health and survival", and "Political empowerment", shows that although no country has yet achieved full gender parity, three Nordic countries have closed at least 80% of their gap: Norway (87.9%), Finland (86.3%) and Sweden (81.5%); and Denmark is not far behind with 78%.
- This report provides objective gender equality data in the Nordic ATMP space for analysis and comparison with other geographies.









### Gender Equality in the Nordic ATMP Space

2023

#### Introduction

#### **Definition**

Gender equality refers to the equal rights, opportunities, and treatment of individuals regardless of their gender identity or expression. It encompasses the fair and unbiased representation, participation, and treatment of people of all genders within societal, professional, and organizational contexts.

Aspects of Gender Equality within this mapping of the ATMP Field:

- Representation and Participation in Clinical Trials: Ensuring equal representation and participation of individuals of all genders in clinical trials for ATMP therapies. This includes addressing barriers that may prevent certain gender groups from participating fully or equally in clinical research.
- Workforce Diversity in ATMP Companies: Promoting gender diversity within ATMP companies at all levels, including leadership positions, research teams, and technical roles. This involves creating inclusive hiring practices, policies, and organizational cultures that support gender equality and diversity.
- Equitable Recognition of Key Opinion Leaders (KOLs): Recognizing and promoting diverse voices and expertise within the field of ATMP, including equitable representation of women and underrepresented genders among key opinion leaders (KOLs). This ensures that diverse perspectives contribute to decision-making processes, research advancements, and industry innovation.
- Therefore, the analysis presented in this report is based on literature data, as well as investigation of ATMP companies and key opinion leaders (KOLs) in the Nordics. In addition, analysis of ATMPs that are approved or in the pipeline is included. Data are divided into three different sections:
  - ATMP Therapies & Clinical Trials
  - ATMP Companies
  - Key Opinion Leaders (KOLs)

Source: Global Gender Gap Report 2023, World Economic Forum, Link

#### **Executive Summary**

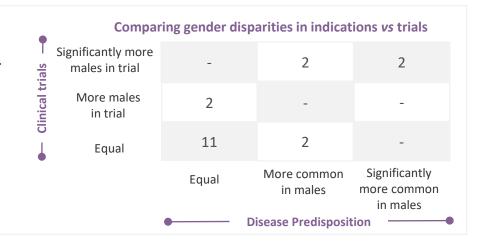
#### **Overall conclusions**

- Despite significant advancements in gender equality in the Nordic countries, as evidenced by the 2023 Global Gender Gap Index, there are still noteworthy differences between the genders.
- In particular, within the ATMP field, there is a considerable gender imbalance in top leadership positions, with a substantial majority of CEOs, C-level executives, and Board of Directors being male. Additionally, among the 50 most relevant KOLs in the industry, about two-thirds are men.
- In the assessment of ATMP-approved treatments and late-stage pipeline, despite many targeted diseases having equal gender prevalence, onethird of these therapies focus on male-related diseases, while none are focused on female-related indications. However, the analysis was based on a limited dataset, and genetic susceptibility in diseases targeted by ATMPs should be considered.
- While there's substantial work remaining, all comparative studies between the Nordics, Europe, the US, or the global scale indicate that the Nordic countries are making significant progress towards a more equitable environment at a faster pace.

#### Disease predisposition & clinical trials

MSC conducted an analysis of EMA-approved ATMP therapies and assets currently in phase 3 development stage, focusing on the gender predisposition in related diseases.

- Out of the 18\* diseases targeted, 6 exhibit a higher incidence in males compared to females.
- None of the 18\* targeted diseases display a predisposition for females.
- Among the 19 pivotal trials, 13 maintained an equal gender distribution, while 4 had significantly more male participants, and 2 enrolled more male patients.



<sup>\*</sup>There are 18 indications targeted by ATMPs. Multiple Myeloma is targeted by both Abecma and Carvytki, is counted twice in the analysis. Tecartus accounts for two indications, targeting both mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL). This results in 19 data points from only 18 indications.



#### **Executive Summary**

#### **ATMP companies in the Nordics**

The analysis of ATMP SME companies in the Nordics brings objective data in relation to distribution in leadership positions:

- 72% of the CEOs are male;
- 67% of the C-level executives are male;
- 78% of the Board of Directors are male;

However, when Nordic ATMP companies are compared with biotech companies in general, the numbers look more optimistic:

- The 28% share of women in the CEO position is higher in ATMP SME companies in the Nordics when compared with the global average in biotech companies, around 20%.
- In relation to the C-level team, the share of female members is 33% in ATMP companies in the Nordics, while biotech in general has 34% of female in executive management positions.



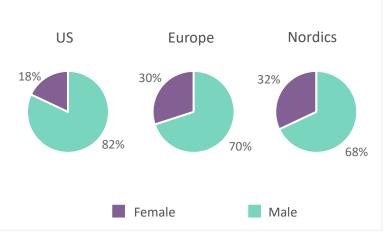
The Nordic countries exhibit a comparatively lower level of gender imbalance, but the analysis reveals a significant disparity in the 50 most relevant ATMP key opinion leaders (KOLs) across each geography:

• 82% of KOLs in the US are male, followed by 70% in Europe and 68% in the Nordics.

Additionally, there is a notable discrepancy in the number of publications as last authors, with males having a significantly higher count.

Overall, across US and Europe, there is a consistent trend of male KOLs receiving higher industry payments and grant funding compared to their female counterparts. The difference is most pronounced in the US and least in Europe.







ATMP Therapies & Clinical Trials



### ATMP Therapies and Clinical Trials Analysis: Methodology and limitations

- MSC conducted an analysis of EMA-approved ATMP therapies and assets currently in the phase 3 development stage, focusing on their respective target indications and gender predisposition in targeted diseases. Additionally, the analysis of their phase 3 pivotal studies and the gender distribution among the trials' participants was performed.
- To categorize these diseases, a prevalence-based classification system was employed, which considers the distribution of cases between genders:
  - If more than 75% of cases predominantly affect one gender, it was categorized it as "significantly more common in males" or "significantly more common in females."
  - Diseases affecting a specific gender in 60-74% of cases were classified as "more common in males" or "more common in females."
  - When one gender was affected in 41-59% of cases, the disease's prevalence was marked as "equal."
  - The analysis could not identify a scientifically based rationale for the distinction between the categories. Therefore, it was chosen to utilize predefined intervals in the distribution of gender to categorize the indications and trials.
- Information on EMA-approved therapies was gathered from the official EMA website (<u>Link</u>) and data on phase 3 assets from the Gene Therapy Report by CVS Health (<u>Link</u>). To establish disease prevalence, relevant scientific publications were used as sources (see appendix).
- It's important to note that the analysis is based on a limited dataset, as only 18 EMA-approved therapies and 20 phase 3 assets were identified.
- Additionally, gender-specific differences in genetic diseases susceptibility might have influenced the results, for instance, chromosome X-linked diseases, which several regenerative medicine assets target, are significantly more often expressed in males.

Note: Gender identities beyond female and male were not considered in the analysis.

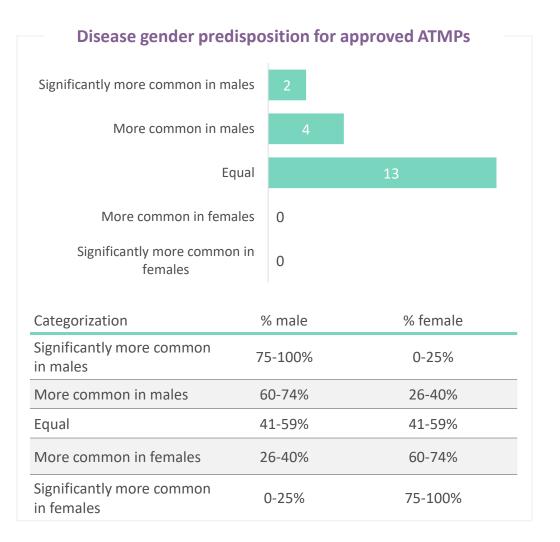


## One-third of the indications targeted by ATMPs are notably more common in males, while none is more common in females

- 13 out of the 18\* indications targeted by EMA-approved ATMPs do not exhibit a gender predisposition and are categorized as having an equal distribution of men and women.
- Two diseases targeted by ATMPs are categorized as being 'significantly more common in males':
  - Hemophilia A, targeted by Roctavian.
  - Hemophilia B, targeted by Hemgenix.
- Four diseases are categorized as 'more common in males':
  - Mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL), targeted by Tecartus.
  - Limbal stem cell deficiency (LSCD), targeted by Holoclar.
  - Spinal Muscular Atrophy Type 1, targeted by Zolgensma.
- LSCD can be and acquired due to chemical and thermal burns unlike other indications analyzed in this report. The more common appearance in males might therefore be lifestyle or occupationrelated rather than based on biological gender.
- None of the ATMP-targeted indications are more common in females.

Note: Gender predisposition in the disease is indiscriminate of disease severity.

Sources: MSC analysis; EMA website. For full sources, see appendix.





<sup>\*</sup>There are 18 indications targeted by ATMPs. Tecartus accounts for two indications, targeting both mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL). Multiple Myeloma is targeted by both Abecma and Carvytki and is counted twice in the analysis. This results in 19 data points from only 18 indications.

# Following the approved ones, most late-stage pipeline therapies have no gender predisposition, while 1/3 target male diseases

- Within the ATMP pipeline, 20 phase 3 candidates were identified.
- Among these candidates, 13 of the 20 assets target indications categorized as 'equal' distribution of men and women.
- 7 assets are targeted toward diseases that fall into the category of 'significantly more common in males':
  - 2 assets are directed at X-linked retinitis pigmentosa.
  - 1 asset targets Duchenne muscular dystrophy.
  - 1 asset targets ornithine transcarbamylase deficiency.
  - 1 asset is directed at hemophilia B.
  - 1 asset targets hemophilia A.
  - 1 asset targets localized prostate cancer.
- All of these 7 diseases, except localized prostate cancer, are X-linked diseases, meaning both males and females are carriers, but the disease is significantly more often expressed in males.
- As many of the phase 3 trials are ongoing, there is no data available for the gender disposition in these trials and analysis was not performed.

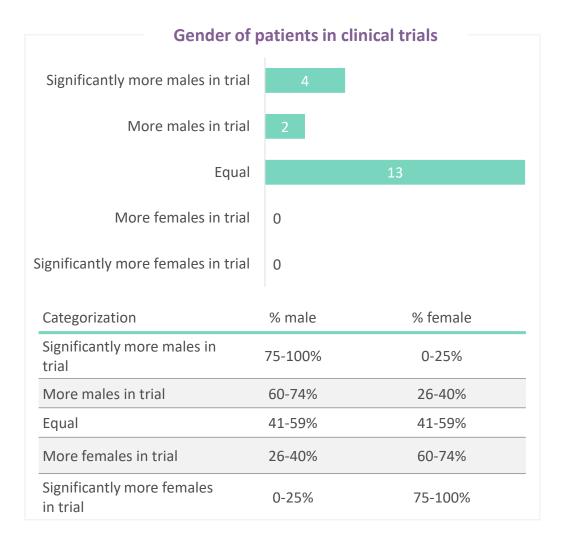
Disease gender predisposition for phase 3 assets Significantly more common in males More common in males Equal More common in females Significantly more common in females Categorization % female % male Significantly more common 75-100% 0-25% in males More common in males 60-74% 26-40% Equal 41-59% 41-59% More common in females 26-40% 60-74% Significantly more common 0-25% 75-100% in females

Note: X-linked diseases are considered to be significantly more common in males due to the impact of the X chromosome, even though both males and females can carry the genetic mutation equally.



### For the approved therapies, 6 of 19 pivotal trials included notably more males, while none included more females

- Focusing on just the pivotal clinical trials for each approved ATMP, analysis shows that 13 of the 19\* pivotal trials exhibit an equal number of males and females participating and are categorized as 'Equal'.
- 4 assets fell into the category 'significantly more males in trial' with over 75% males participating in their pivotal trials:
  - Holoclar, targeting limbal stem cell deficiency
  - Tecartus, in the trial of mantle cell lymphoma
  - Roctavian, targeting hemophilia A
  - Hemgenix, targeting hemophilia B
- 2 drugs were categorized as 'more males in trial':
  - Yescarta, targeting large B-cell lymphoma
  - Strimvelis, targeting severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)

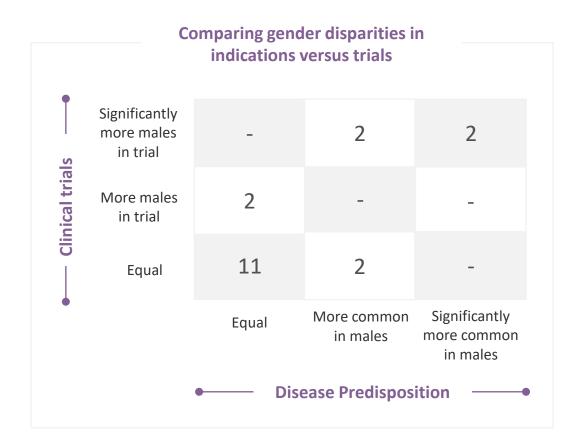




<sup>\*</sup>There are 18 indications targeted by ATMPs. Tecartus accounts for two indications, targeting both mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL). When an ATMP has multiple clinical trials in the same indication reported as influential in the EMA report, the participants are added together and counted as one study. Multiple Myeloma which is targeted by both Abecma and Carvytki, is counted twice in the analysis. This results in 19 data points from only 18 indications.

## A disease gender predisposition does not mean the trial will be equally gender distributed

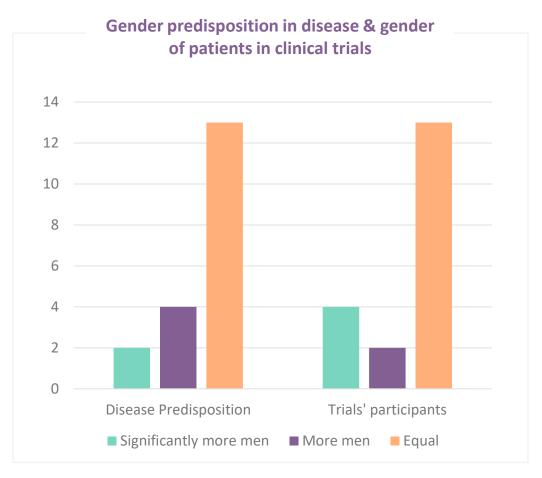
- Gender distribution in clinical trials commonly mirrors disease predisposition, but disparities are not uncommon.
- Roctavian and Hemgenix, marketed for hemophilia A and hemophilia B, consistently were categorized as 'significantly more common in males' in both disease predisposition and clinical trials.
- Two studies had a distribution that fell into the 'significantly more common in male patients' category, despite the disease only being in the category 'more common in males'.
  - Tecartus study in mantle cell lymphoma had 84% male patients, despite a 70% male predisposition.
  - Holoclar study for limbal stem cell deficiency was 77% male, while the disease had a 70% male predisposition.
- Yescarta and Strimvelis studies had higher male proportions (66% and 60%) despite no gender predisposition in the targeted diseases.
- The pivotal studies of Zolgenma and Tecartus in Spinal Muscular Atrophy type 1 and Acute lymphoblastic leukemia did not consist of more males even though the diseases are more common in males.





## Conclusion: ATMPs often target diseases with male predisposition, but gender prevalence in most indications and trials is equal

- Overall, data suggests that ATMPs predominantly address conditions with an equal gender predisposition. However, when a gender predisposition exists, it tends to be toward the male population.
- Out of the 18\* diseases targeted, 6 exhibit a higher prevalence in males compared to females.
- None of the 18\* targeted diseases display a predisposition for females.
- Among the 19 pivotal trials, 13 maintained an equal gender distribution, while 4 featured significantly more male participants, and 2 enrolled more male patients.
- None of the 19 pivotal trials had more female participants.
- While the patient distribution in trials often reflects the disease predisposition there are disparities where trials patient distribution does not match the disease predisposition.
- Four studies recorded a greater proportion of male patients in comparison to the disease's natural predisposition, while two studies observed a higher proportion of female participants relative to disease prevalence.



<sup>\*</sup>There are 18 indications targeted by ATMPs. Tecartus accounts for two indications, targeting both mantle cell lymphoma (MCL) and acute lymphoblastic leukemia (ALL). Multiple Myeloma is targeted by both Abecma and Carvytki and is counted twice in the analysis. This results in 19 data points from only 18 indications.

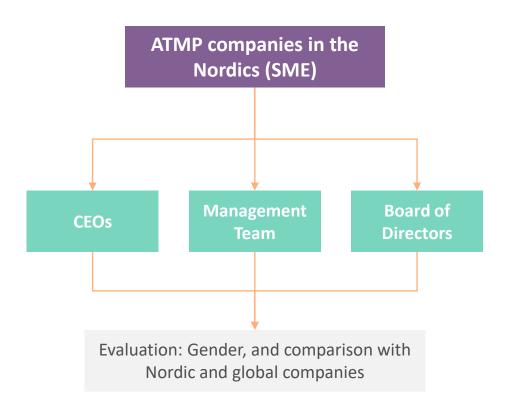


ATMP Companies



### The ATMP Companies Analysis: Methodology and limitations

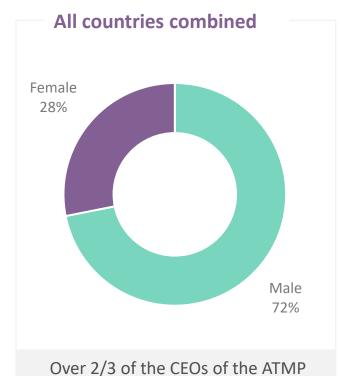
- MSC Nordics conducted an analysis of the the gender of CEOs, C-level Executive Team, and Board of Directors (BoD) of the Small and Medium-sized Enterprises (SMEs) focused on regenerative medicine in the four Nordic countries: Sweden, Denmark, Finland and Norway.
- The SMEs selected were companies based in the Nordics developing ATMPs, with currently no marketed products.
   Previous MSC analysis, GlobalData, Datamonitor, and the Nordic Life Sciences Database were utilized to identify the companies.
- Information about the companies' CEOs, management team, and BoD was collected through the companies' websites.
- Data from this analysis was compared with numbers from larger public companies in the same geographies, with data from European Institute for Gender Equality; and Biotech and companies in general, with data from the BIO Industry Analysis Report.
- A full list of the companies included is available in appendix.





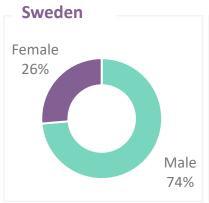
### Analysis of the ATMP companies identified in the Nordics show that 72% of the CEOs are male

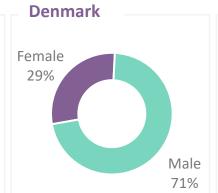
#### **CEOs of ATMP companies by gender**

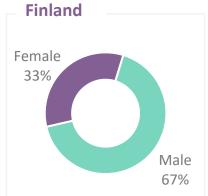


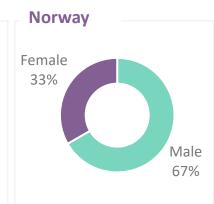
companies identified in the Nordic

countries are male









Country	Female CEO	Male CEO	N/A	Total
Denmark	2	5	2	9
Finland	1	2	0	3
Norway	1	2	1	4
Sweden	5	14	1	20
Total	10	23	4	37

From the four Nordic countries, Sweden has the majority of ATMP companies, and 74% of the CEOs are male in the country.

N/A: Information not available. The female/male percentages were calculated excluding the companies that don't have information available. It is important to note that numbers from Finland are based on 3 companies only, and Norway numbers are based on 4 ATMP companies.

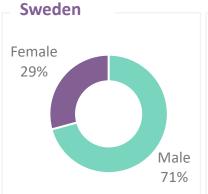


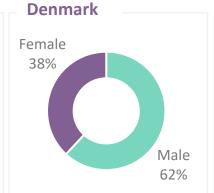
## The differences in gender representation are similar in management teams, and only 33% of the C-level executives are female

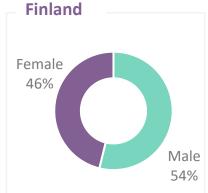
#### C-level management teams of ATMP companies by gender

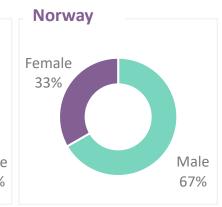












Country	Male executive	Female executive
Denmark	13	8
Finland	7	6
Norway	6	3
Sweden	46	19
Total	72	36

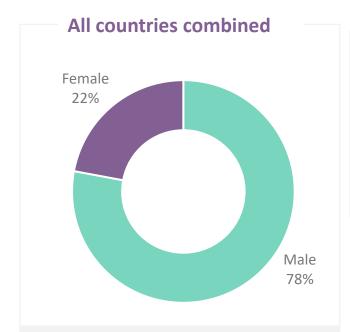
The differences in gender representation remain significant in the C-level management teams from the Nordics, particularly in Sweden, Denmark, and Norway.

Due to methodology simplification and standardization, management executives were considered the in C-level positions only, e.g., CTO, CFO, CMO, CSO, etc.

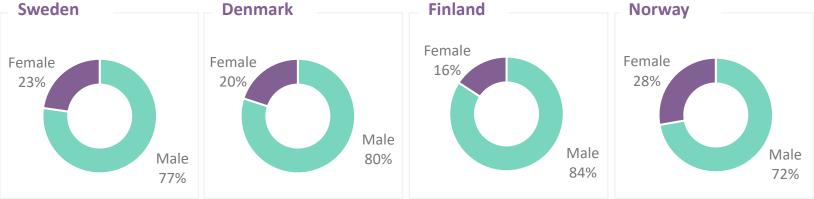


### Almost 80% of the board of directors in the ATMP companies identified in the Nordic countries are male

#### Board of directors of ATMP companies by gender



Almost 80% of the board of directors in the ATMP companies identified in the Nordic countries are male.

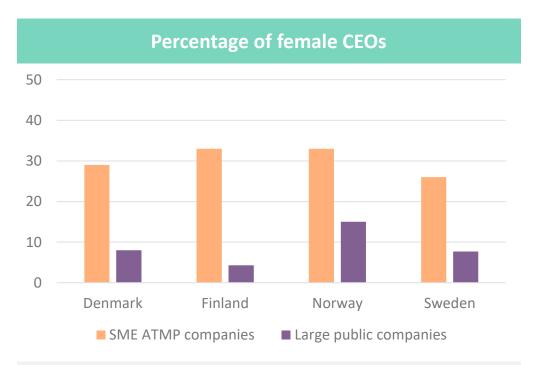


Country	Male board member	Female board member
Denmark	16	4
Finland	16	3
Norway	13	5
Sweden	68	20
Total	113	32

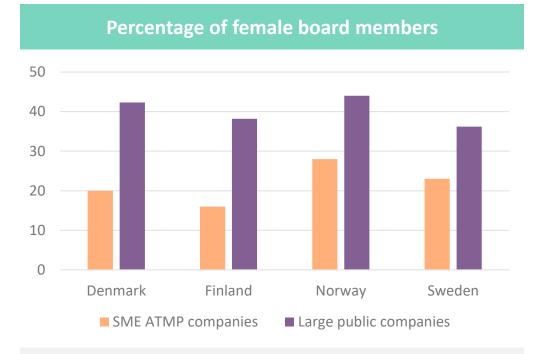
The proportion of male board members varies from 72% to 84% among the four Nordic countries.



### The share of female CEOs is significantly higher in SME ATMP companies, but large companies have more female board members



The share of women in the CEO position is significantly higher in ATMP companies in the Nordics (SME), when compared with larger public companies in the same geographies.



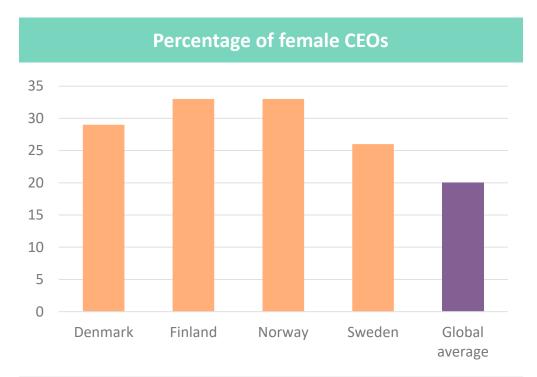
In relation to the board of directors, the share of female members is higher among large public companies from various sectors, when compared with ATMP companies. Some countries' legislations mandate women on corporate boards through gender quotas.

Notes: It is important to note that numbers from Finland and Norway are based on three and four ATMP companies only.

Publicly listed means that the shares of the company are traded on each country's stock exchange, including all sectors. The "largest" companies are taken to be the members (max.50) of the primary blue-chip index, which is an index maintained by the stock exchange and covers the largest companies by market capitalization and/or market trades.

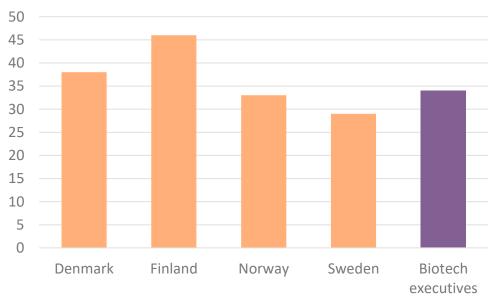


## The share of women in the CEO position is higher in SME ATMP companies in the Nordics than the global average in biotech



The share of women in the CEO position is higher in ATMP companies in the Nordics (SME) when compared with the global average in biotech companies.

### Percentage of female executive members

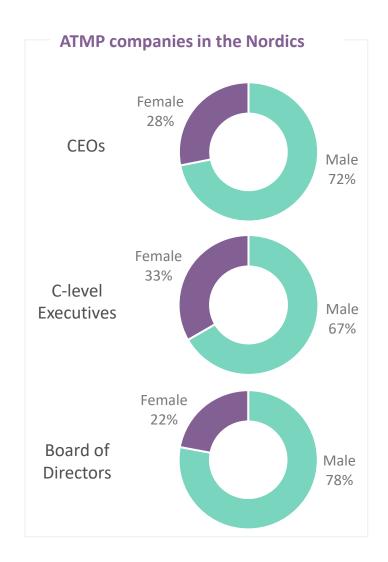


The female representation among SME ATMP companies C-level executives is comparable with biotech companies across the globe. Finland stands out with the country with the higher number of female executives.

Notes: It is important to note that numbers from Finland and Norway are based on three and four ATMP companies only. Biotech companies used as comparison include those headquartered across the globe, with 85% based in the US and 8% in Europe.



## Conclusion: Nordic ATMP companies show progress in leadership gender equality compared to biotech sector and large companies



- Gender bias in leadership positions is not a new topic, and the analysis of ATMP companies in the Nordics brings objective data for comparison:
  - 72% of the CEOs of the ATMP companies identified in the Nordic countries are male
  - 67% of the C-level executive team are male
  - 78% of the Board of Directors are male
- However, when Nordic ATMP companies are compared with biotech companies in general, the numbers look slightly more optimistic:
  - The 28% share of women in the CEO position is higher in ATMP companies in the Nordics (SME) when compared with the global average in biotech companies, around 20%.
  - In relation to the corporate management team (C-level), the share of female members is 33% in ATMP companies in the Nordics, while biotech in general has 34%.
- When it comes to board of directors, usually senior positions, the small ATMP companies in the Nordics present a lower percentage of female members, 22%, than large public companies from each country analyzed (36-44%).

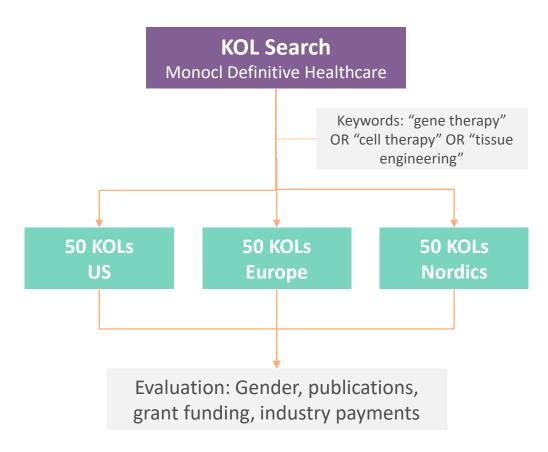


Key Opinion Leaders (KOLs)



### The Key Opinion Leader Analysis: Methodology and limitations

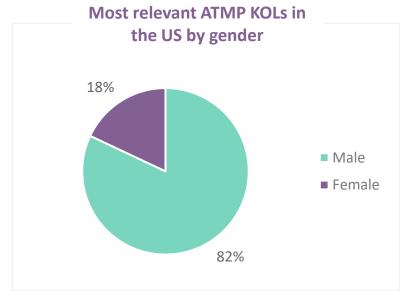
- MSC Nordics conducted an analysis of the most relevant key opinion leaders (KOLs) in ATMP, based on the Monocl Definitive Healthcare platform.
- The keywords utilized to filter the experts were: "gene therapy" OR "cell therapy" OR "tissue engineering".
- The results were sorted by relevance.<sup>1</sup>
- The fifty most relevant KOLs' profiles in the following geographies were divided based on our analysis of gender, and the platform number of publications, grant funding, and industry payments:
  - US
  - Europe
  - Nordic countries: Sweden, Denmark, Finland, and Norway



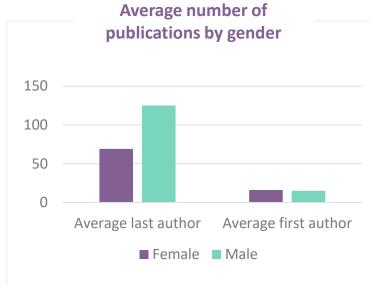
1. The relevance score takes several factors into consideration, e.g., the number of publications, journal impact, clinical trials, meeting presentations, and grant payments generated by each expert within the specific search, etc. It is important to acknowledge that Monocl, as any database, may have inherent limitations. While Monocl is a valuable resource for identifying influential figures, it may inadvertently reflect societal biases, including gender bias.



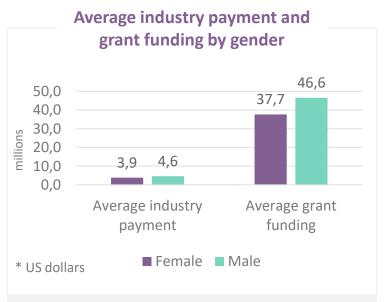
### The majority of the most relevant KOLs in the US in ATMP are male; and male KOLs have more publications and grant funding



Out of the 50 KOL considered the most relevant experts in ATMP in the US, there is an important gender disparity: 41 are male and only 9 are female



While the average number of publications as first author is equal between male and female KOLs, the number of publications as last author is much higher among males

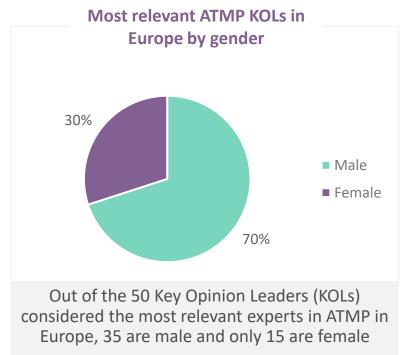


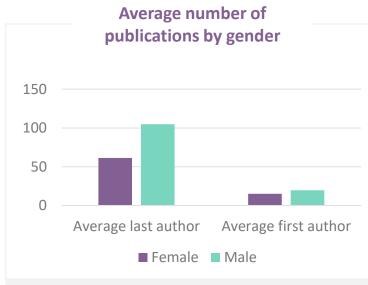
The average KOLs industry payment is 3.9M for female KOLs and 4.6M for males, and female grant funding is on average 37.7M, while male grants are 46.6M.

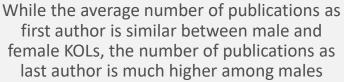
Note: The search was made through Monocl Definitive Healthcare platform in Oct 2023, including the keywords "gene therapy" OR "cell therapy" OR "tissue engineering", filtering the geographies of interest, and sorting by relevance.

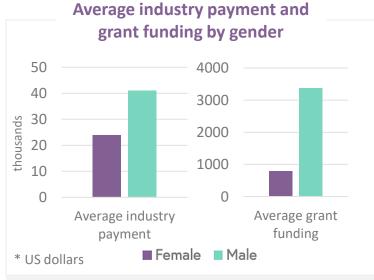


### European KOLs show less gender inequality than American counterparts, yet only 30% of relevant KOLs in Europe are female







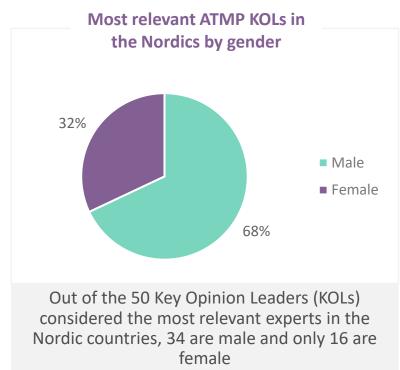


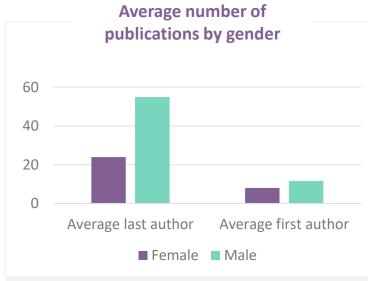
The average KOLs industry payment is 24 thousand for female KOLs and 41 thousand for males; average female grant funding is 0.79M, while male grants are 3.4M.

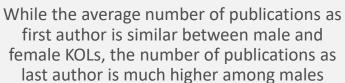
Note: The search was made through Monocl Definitive Healthcare platform in Oct 2023, including the keywords "gene therapy" OR "cell therapy" OR "tissue engineering", filtering the geographies of interest, and sorting by relevance.

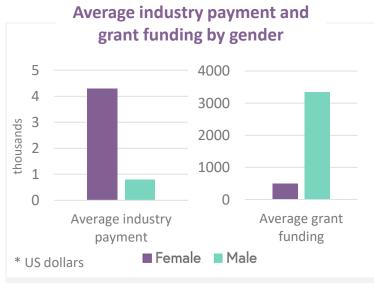


### The number of female ATMP KOLs is slightly higher in the Nordics than in Europe as a whole, but male KOLs receive higher grants







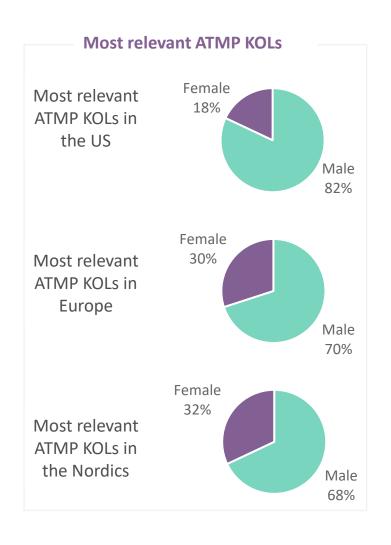


The average KOLs industry payment is 4.3 thousand for female KOLs and 0.8 thousand for males; average female grant funding is 0.49M, while male grants are 3.3M.

Note: The search was made through Monocl Definitive Healthcare platform in Oct 2023, including the keywords "gene therapy" OR "cell therapy" OR "tissue engineering", filtering the geographies of interest, and sorting by relevance.



### Conclusion: Gender disparity among Nordic ATMP KOLs is substantial, but it is less intense than the US and Europe



- While the Nordic countries exhibit a comparatively lower level of gender imbalance, the analysis reveals a significant disparity in the most relevant ATMP key opinion leaders across all geographies:
  - Specifically, 82% of KOLs in the US are male, followed by 70% in Europe and 68% in the Nordics.
- Although male and female KOLs across all geographies (the US, Europe, and the Nordic countries) have a similar average number of publications as first authors, there is a notable discrepancy in the number of publications as last authors, with males having a significantly higher count.
- Across US and Europe, there is a consistent trend of male KOLs receiving higher industry
  payments and grant funding compared to their female counterparts. The difference is most
  pronounced in the US and least pronounced in Europe.
- In the Nordics, female KOLs received higher industry payments compared to male, which is a deviation from the trends observed in the US and Europe; however, male KOLs received higher grant funding compared to females.
- Industry and grant payments are overall significantly lower in Europe when compared to the US; and industry payments are even lower in the Nordic countries.
- It's important to acknowledge potential biases in the data, including discussions about gender bias in AI tools (which reflect existing biases in society), and may influence the presentation of male KOLs as more relevant.







### Diseases targeted by ATMPs and their predisposition for gender

Indication	Targeted by therapy	% Male patients	% Female patients	Gender predisposition	Source
Spinal Muscular Atrophy Type 1 (SMA type 1)	Zolgensma	61%	37%	More common in males	<u>Link</u>
Limbal stem cell deficiency (LSCD)	Holoclar	70%	30%	More common in males	<u>Link</u>
Melanoma	Imlygic	56%	44%	Equal	<u>Link</u>
Adenosine deaminasedeficient severe combined immunodeficiency (ADA-SCID)	Strimvelis	Equal*	Equal*	Equal	No difference found
Cartilage defects in the knee	Spherox	Equal*	Equal*	Equal	<u>Link</u>
Complex perianal fistulas in adult patients with non- active/mildly active luminal Crohn's disease	Alofisel	Equal*	Equal*	Equal	<u>Link</u>
Large B-cell lymphoma (DLBCL)	Yescarta	56%	44%	Equal	<u>Link</u>
Follicular lymphoma (FL)	Kymriah	49%	51%	Equal	<u>Link</u>
Retinal dystrophy with biallelic RPE65 mutations	Luxturna	Equal*	Equal*	Equal	<u>Link</u>
Metachromatic leukodystrophy (MLD)	Libmeldy	Equal*	Equal*	Equal	<u>Link</u>
Mantle cell lymphoma (MCL)	Tagantus	72%	28%	More common in males	<u>Link</u>
Acute lymphoblastic leukemia (ALL)	Tecartus	65%	35%	More common in males	<u>Link</u>
Multiple musleme (MANA)	Abecma	58%	42%	Faual	Link
Multiple myeloma (MM)	Carvykti	38%	42%	Equal	<u>Link</u>
Large B-cell lymphoma (LBCL)	Breyanzi	56%	44%	Equal	<u>Link</u>
AADC deficiency	Upstaza	Equal*	Equal*	Equal	<u>Link</u>
Hemophilia A	Roctavian	97%	3%	Significantly more common in males	<u>Link</u>
Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)	Ebvallo	Equal*	Equal*	Equal	<u>Link</u>
Hemophilia B (congenital Factor IX deficiency)	Hemgenix	100%	0%	Significantly more common in males	<u>Link</u>

<sup>\*</sup>The disease was found to have equal gender predisposition, but no specific percentage of male/female was identified



### List of pivotal clinical trials with ATMPs and participants by gender

ATMP Therapy	Indication	% Male patients (Nr Male patients)	% Female patients (Nr Male patients)	Gender distribution in clinical trials	Source
Zolgensma	Spinal Muscular Atrophy Type 1 (SMA type 1)	45% (10)	55% (12)	Equal	<u>Link</u>
Holoclar	Limbal stem cell deficiency (LSCD)	77% (80)	23% (24)	Significantly more males in trial	<u>Link</u>
Imlygic	Melanoma	42% (186)	57% (250)	Equal	<u>Link</u>
Strimvelis	Adenosine deaminasedeficient severe combined immunodeficiency (ADA-SCID)	60% (11)	40% (7)	More males in trial	<u>Link</u>
Spherox	Cartilage defects in the knee	52% (55)	48% (50)	Equal	<u>Link</u>
Alofisel	Complex perianal fistulas in adult patients with non-active/mildly active luminal Crohn's disease	54% (114)	46% (98)	Equal	<u>Link</u>
Yescarta	Large B-cell lymphoma (DLBCL)	66% (237)	34% (122)	More males in trial	<u>Link</u>
Kymriah	Follicular lymphoma (FL)	56% (99)	44% (77)	Equal	<u>Link</u>
Luxturna	Retinal dystrophy with biallelic RPE65 mutations	42% (13)	58% (18)	Equal	<u>Link</u>
Libmeldy	Metachromatic leukodystrophy (MLD)	55% (16)	45% (13)	Equal	<u>Link</u>
	Mantle cell lymphoma (MCL)	84% (62)	16% (12)	Significantly more males in trial	<u>Link</u>
Tecartus	Acute lymphoblastic leukemia (ALL)	58% (41)	42% (30)	Equal	<u>Link</u>
Abecma	Multiple myeloma (MM)	59% (82)	41% (58)	Equal	<u>Link</u>
Carvykti	Multiple myeloma (MM)	58% (122)	42% (88)	Equal	<u>Link</u>
Breyanzi	Large B-cell lymphoma (LBCL)	57% (105)	43% (79)	Equal	<u>Link</u>
Upstaza	AADC deficiency	59% (13)	41% (9)	Equal	<u>Link</u> , <u>Link</u>
Roctavian	Hemophilia A	100% (134)	0% (0)	Significantly more males in trial	<u>Link</u>
Ebvallo	Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)	50% (15)	50% (15)	Equal	<u>Link</u>
Hemgenix	Hemophilia B (congenital Factor IX deficiency)	100% (54)	0% (0)	Significantly more males in trial	<u>Link</u>



## Diseases targeted by phase 3 ATMPs and their predisposition for gender (1/2)

Indication	Targeted by therapy	% Male patients	% Female patients	Gender predisposition	Source
Sickle cell disease	Exagamglogene autotemcel	Equal*	Equal*	Equal	<u>Link</u>
Sickle cell disease	Lovotibeglogene autotemcel	Equal*	Equal*	Equal	<u>Link</u>
Relapsed or refractory multiple myeloma	Abecma (Idecabtagene vicleucel)	58%	42%	Equal	<u>Link</u>
Relapsed or refractory multiple myeloma	Carvykti (ciltacabtagene autoleucel)	58%	42%	Equal	<u>Link</u>
Transfusion- dependent beta thalassemia	Exagamglogene autotemcel	Equal*	Equal*	Equal	<u>Link</u>
Hemophilia B	Fidanacogene elaparvovec	100%	0%	Significantly more common in males	<u>Link</u>
Metachromatic leukodystrophy	Libmeldy (Atidarsagene autotemcel)	Equal*	Equal*	Equal	<u>Link</u>
Recessive dystrophic epidermolysis bullosa	dabocemagene autoficel	Equal*	Equal*	Equal	<u>Link</u>
Recessive dystrophic epidermolysis bullosa	EB101	Equal*	Equal*	Equal	<u>Link</u>



<sup>\*</sup>The disease was found to have equal gender predisposition, but no specific percentage of male/female was identified

## Diseases targeted by phase 3 ATMPs and their predisposition for gender (2/2)

Indication	Indication Targeted by therapy		% Female patients	gender predisposition	Source
Diabetic peripheral neuropathy	Engensis (donaperminogene seltoplasmid)	Equal*	Equal*	Equal	<u>Link</u>
Mucopolysaccharidosis type IIIA	UX111	Equal*	Equal*	Equal	<u>Link</u>
X-linked retinitis pigmentosa	Botaretigene sparoparvovec	54%	46%	Significantly more common in males	<u>Link</u>
X-linked retinitis pigmentosa	Laruparetigene zosaparvovec	54%	46%	Significantly more common in males	<u>Link</u>
Duchenne muscular dystrophy	Fordadistrogene movaparvovec	100%	0%	Significantly more common in males	<u>Link</u>
Glycogen storage disease type 1a	Pariglasgene brecaparvovec	Equal*	Equal*	Equal	<u>Link</u>
Hemophilia A	Giroctocogene fitelparvovec	97%	3%	Significantly more common in males	<u>Link</u>
Ornithine transcarbamylase deficiency	Avalotcagene ontaparvovec	Equal*	Equal*	Equal	<u>Link</u>
Knee osteoarthritis	Invossa	53%	47%	Significantly more common in males	<u>Link</u>
Localized prostate cancer	ProstAtak (aglatimagene besadenovec)	Equal*	Equal*	Equal	Only in males
Neovascular (wet) age-related macular degeneration	RGX314	45%	55%	Significantly more common in males	<u>Link</u>



<sup>\*</sup>The disease was found to have equal gender predisposition, but no specific percentage of male/female was identified

# List of Nordic companies identified, including CEO, BoD, and C-level management by gender (1/2)

Name	Country	CEO	Board: male	Board: female	C-level team: Male	C-level team: Female
Alder Therapeutics	Sweden	Male	0	0	3	0
Amniotics	Sweden	Male	4	1	3	0
Anocca	Sweden	Male	6	1	8	0
Asgard Therapeutics	Sweden	Female	7	0	0	1
Aurealis pharma	Finland	Male	5	0	3	0
Blue Cell Therapeutics	Denmark	Male	2	0	2	0
BOOST Pharma	Denmark	Male	1	2	2	2
Carry Genes	Sweden	Male	4	1	1	0
Cbio	Denmark	Male	1	1	2	1
Cell2Cure	Denmark	Male	0	0	1	0
Cline Scientific AB	Sweden	Female	4	1	2	0
CombiGene	Sweden	Male	3	2	1	2
Cytovac	Denmark	N/A	5	0	1	1
Elicera	Sweden	Male	2	3	3	0
Ilya Pharma	Sweden	Female	1	1	0	1
Immunicum/Mendus	Sweden	Male	4	2	4	1
Lokon Pharma AB	Sweden	Female	0	2	0	1
Neogap (TCER AB)	Sweden	Male	4	2	2	3

Data from the companies' website from November 2023. Unikum Therapeutics' website could not be identified; therefore, the company data is based on its LinkedIn page.



# List of Nordic companies identified, including CEO, BoD, and C-level management by gender (2/2)

Name	Country	CEO	Board: male	Board: female	C-level team: Male	C-level team: Female
Nextcell Pharma AB	Sweden	Male	3	1	2	1
Pancryos	Denmark	Female	0	0	0	1
PokeAcell	Denmark	Female	0	0	1	2
Procella/SmartCella Therapeutics	Sweden	Male	3	0	3	1
Sense Biopharma	Norway	N/A	3	1	0	0
Sinfonia Biotherapeutics	Sweden	Male	1	0	5	0
SNIPR Biome	Denmark	Male	5	1	3	0
Stem Sight	Finland	Female	6	2	1	4
StromaBio	Sweden	N/A	0	0	0	2
Takura	Sweden	Male	6	1	1	4
Targovax (Circio)	Norway	Male	3	1	3	0
Thelper AS	Norway	Female	2	3	0	0
TILT Biotherapeutics Ltd	Finland	Male	5	1	3	2
Unikum Therapeutics	Denmark	N/A	2	0	1	1
Verigraft	Sweden	Male	6	1	3	0
Xintela	Sweden	Female	5	0	1	1
XNK Therapeutics	Sweden	Male	5	1	4	1
Zelluna	Norway	Male	5	0	3	3

Data from the companies' website from November 2023. Unikum Therapeutics' website could not be identified; therefore, the company data is based on its LinkedIn page.

