Comparing early access to unlicensed medicines in UK, France, and Sweden: Consistent principles but inconsistent outputs

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Background

- Early Access Programs allow cohorts of patients with severe diseases with no therapeutic alternatives, access to unlicensed therapies in clinical development
- The schemes in the UK, France and Sweden are the:
  - UK - Early Access to Medicines Schemes (EAMS),
  - France - cohort Temporary Authorization for Use (cohort-ATU)
  - Sweden - Compassionate Use Program (CUP)
- Their main distinction relates to reimbursement: the cohort-ATU allows manufacturers to charge whereas under EAMS and CUP the manufacturer must provide for no charge
- This research compares the drugs available under EAMS, cohort-ATU, and CUP schemes

Methods

- Medicines accepted for use under EAMS, CUP and cohort-ATUs were identified from the relevant websites and key information was extracted (01/01/2015-27/12/2018)

Results

- 22 drug:indication pairings have been accepted onto EAMS
  - 86% (19/22) have expired after a mean 95 days (range:19-308). 59% (13/22) were for oncology and 18% (4/22) had orphan designations
- 49 drug:indication pairings attained cohort-ATUs. 65% (32/49) closed after a mean of 261 days (range:61-1001 days)
  - 41% (20/49) were for oncology and 35% (17/49) had orphan designations
- 7 drug:indication pairings have been accepted onto CUP
  - 56% (4/7) were for oncology and 14% (1/7) had orphan designations (Table 1)

Table 1: Comparison of drug: indication pairings accepted onto EAMS vs. Cohort-ATU vs. CUP (01/01/2015-27/12/2018)

	Cohort-ATUs	EAMS	CUP
No. accepted	49	22	16
Oncology indications	41%	59%	75%
Orphan Indications	35%	18%	19%
Expired	65%	86%	81%
Average time open	261 days	95 days	336 days

- Only 2 drug:indication pairings (patisiran and venetoclax) were accepted onto all three of cohort-ATUs, CUP, and EAMS
  - Despite similar start dates, time on cohort-ATU scheme was numerically higher than on EAMS and CUP
  - (mean: 114 vs. 94 vs. 75 days) (Table 2)

Table 2: Comparison of drug: indication pairings accepted onto both EAMS, CUP, and Cohort-ATUs (01/01/2015-27/12/2018)

Drugs in both EAMS and Cohort-ATU		Patisiran	Venetoclax
Indication		hATTR	CLL
EC-approval date		27/08/18	04/12/16
Cohort-ATU	**Start date**	21/06/18	22/08/16
	End date	N/A	N/A
	Time on (days)	N/A	N/A
	Time from EC-approval to end (days)	N/A	N/A
EAMS	**Start date**	02/08/18	23/08/16
	End date	27/08/18	05/12/16
	Time on (days)	26	104
	Time from EC-approval to end (days)	0	1
CUP	**Start date**	16/05/18	19/10/16
	End date	27/08/18	04/12/16
	Time on (days)	103	46
	Time from EC-approval to end (days)	0	0

Conclusion

- The EAMS, CUP and cohort-ATU schemes appear to attract a largely distinct group of therapies
- The cohort-ATU scheme has attracted over double and triple the drug:indication pairings versus the EAMS and CUP respectively, which may be driven by the
  - ability to charge (which is not allowed for therapies in the CUP and EAMS, but is allowed for the cohort-ATU scheme)
  - ability to formally extend beyond EC-approvals, preventing any gap in patient access between EC-approval and reimbursement decisions (which is not allowed for therapies in the CUP and EAMS, but is allowed for the cohort-ATU scheme)
- However, the TLV recently introduced a temporary reimbursement application to cover for this gap, primarily for patients already initiated on treatment. This application is simplified, for example there is no need to justify the price with health economic models

Abbreviations: ATU: Temporary Authorizations for Use; CLL: Chronic lymphocytic leukemia; EAMS: Early Access To Medicines Scheme; EC: European Commission; hATTR: Hereditary transthyretin amyloidosis; PIM: Potentially Innovative Medicine