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Life Science

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弁護士 近藤 純一 / 弁護士 淺井 茉里菜 / 弁護士 藏野 舞 / 弁護士 横田 瑛弓

Contents

Ⅰ. 改正臨床研究法等の施行

再生医療等の安全性の確保等に関する法律及び臨床研究法の一部を改正する法律が 2024 年 6 月 14 日に公布され、2025 年 5 月 31 日に施行されます[1]。また、これに伴い臨床研究法施行規則等も改正され、同日施行されます。本

1 以下、改正前の臨床研究法及び臨床研究法施行規則をそれぞれ「改正前臨床研究法」、「改正前施行規則」といい、

稿では、今回の改正のうち企業との関係で影響が大きいと考えられる変更を取り上げることとしました。

1. 観察研究に関する臨床研究法の適用

改正前臨床研究法では、臨床研究法の適用対象となる「臨床研究」について、医薬品等を人に対して用いることにより、当該医薬品等の有効性又は安全性を明らかにする研究のうち、①研究の目的で検査、投薬その他の診断又は治療のための医療行為の有無及び程度を制御することなく、患者のために最も適切な医療を提供した結果としての診療情報又は試料を利用する研究（いわゆる観察研究）、②治験、③製造販売後調査等、④医療機器の認証基準適合性に関する情報収集のために行う試験を除くものと定義[2]されてきました。今回の改正では、この「臨床研究」の定義に「当該医薬品等を人の疾病の診断、治療若しくは予防のため又は人の身体の構造若しくは機能に影響を及ぼすために用いる場合において、当該医薬品等の有効性又は安全性を明らかにするために追加的に必要となる検査その他の行為（当該人の心身に著しい負担を与えるものとして厚生労働省令で定めるものに限る。）を行うものを含む。」との文言が追加され、いわゆる観察研究であっても、研究目的で研究対象者に著しい負担を与える検査などを行う場合には臨床研究法の適用対象とされました[3]。

また、改正後施行規則では、「人の心身に著しい負担を与えるもの」には「臨床研究の対象者に対して行われる検査その他の行為であって、当該行為が行われた場合における重大な疾病、障害若しくは死亡若しくは感染症その他の臨床研究の安全性に関わる事象の発生頻度又は心身の苦痛若しくは負担の程度が、通常行われる検査その他の行為と比して相当程度高いと認められるもの」が該当すると規定[4]されました。

【改正後の臨床研究法の対象範囲】

医薬品等の使用	検査等	臨床研究法の対象か否か
1．**研究目的で**医薬品等を使用する場合	（内容問わず）	**対象**
2．**通常の医療の提供として**医薬品等を使用する場合	研究目的で研究対象者に著しい負担を与える検査等を通常の医療に追加して行う場合 例：骨髄穿刺、造影剤を使用するCT検査など （厚生科学審議会の意見を聴いて厚生労働省令等で定める）	**対象**
	上記以外の検査等を通常の医療に追加して行う場合	**対象外**
	通常の医療に必要な範囲の検査等のみ （研究目的の検査等は行わない）	**対象外**

（出典：https://www.mhlw.go.jp/content/10800000/001264187.pdf　6項）

2. 適応外薬に関する特定臨床研究の適用範囲

改正前臨床研究法では、臨床研究のうち、①医薬品等製造販売業者又はその特殊関係者から研究資金等の提供を受けて実施するもの並びに②未承認の医薬品等を用いるもの及び医薬品等の適応外使用を行うものを「特定臨床研究」とし、臨床研究実施基準の遵守や実施計画の提出を義務付けていました[5]。そして、適応外使用を行う場合には、

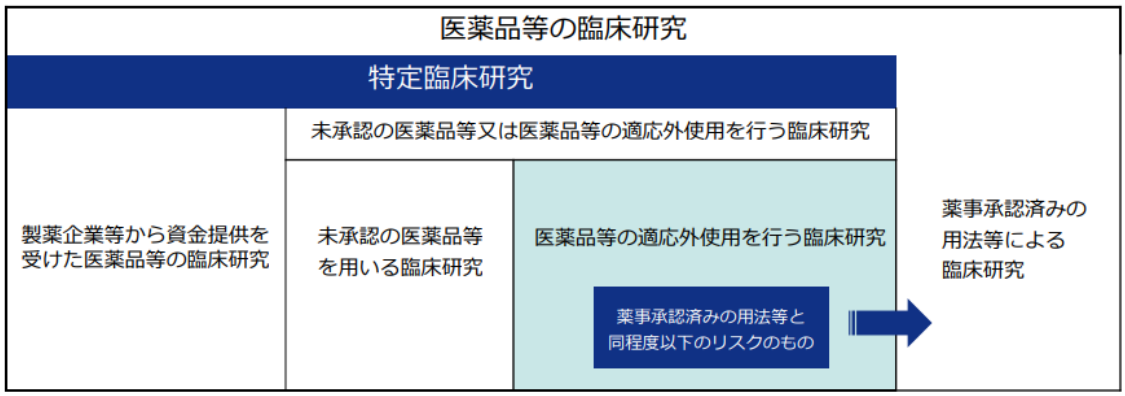
改正後の臨床研究法及び臨床研究法施行規則をそれぞれ「改正後臨床研究法」、「改正後施行規則」といいます。
2 改正前臨床研究法 2 条 1 項、同法施行規則 2 条各号
3 改正後臨床研究法 2 条 1 項
4 改正後施行規則 2 条の 2
5 改正前臨床研究法 2 条 2 項、4 条 2 項、5 条

実際の臨床研究における医薬品等の使用法が承認された用法、用量、効能及び効果と少しでも異なる場合には一律に特定臨床研究に該当するとされていたため、想定される研究対象者へのリスクに比して、研究者に課される負担が大きいとの意見がありました。このような意見を受け、今回の改正では、研究対象者の生命及び健康へのリスクが薬事承認済みの用法等による場合と同程度以下の場合、特定臨床研究には該当しないことが規定されました[6]。

そのうえで、改正後施行規則において、以下の使用方法等についてはリスクが低く特定臨床研究に該当しないことが規定[7]されました（但し、日本国内において、診療等に用いられた実績が乏しい又は保健衛生上の危害が生じている用法等を除く。）。

①医学関連学会が適切な診療等の実施に係る指針の公表等によりその実施を推奨するもの

②その効能・効果・性能が薬事承認の範囲内であり、研究対象者に対する有効性及び安全性が認められるもの



（出典：https://www.mhlw.go.jp/content/10808000/001354318.pdf　2 頁）

3.「統括管理者」の導入

改正前施行規則においては、研究の実施に伴う各対応が研究責任医師に委ねられており[8]、多施設共同研究の場合、監査、モニタリング、疾病等報告等の一部事項について参加医療機関毎に置かれた各責任医師が対応していたため、煩雑であり、同一研究内でも因果関係の判断や各手続きが異なる等の指摘がありました。そこで、国際的な潮流も踏まえ、今回の改正では、新たに、臨床研究全体を統括する統括管理者を設置し、従来研究責任医師に課せられていた責務のうち、研究の計画・運営の責任に関する責務を担うこととされました。多施設共同研究においても、一の臨床研究である場合には一の統括管理者を置く[9]こととし、法人及び団体も統括管理者になることができる[10]ものとされました。

4. 疾病等報告の範囲等について見直し

改正前施行規則においては、既知かつ重篤な疾病等の報告期日について、医薬品等が未承認・適応外の場合は定期報告（1 年に 1 回程度）で足りた一方、既承認の場合は 30 日以内の報告が求められていたため、想定されるリスクと報告期日が相反しているとの指摘がありました。そこで今回の改正では、未承認・適応外の医薬品等の臨床研究において既知の重篤な疾病等を CRB に報告する期限については、原則 30 日以内とされた一方、既承認の医薬品等の臨床研究で発生する既知の副作用は、重篤なものも含めて通常の診療においても起こりうる事象であることを踏

6 改正後臨床研究法 2 条 2 項 2 号
7 改正後施行規則 5 条
8 改正前臨床研究法 14 条、同施行規則 17 条、18 条等
9 改正後施行規則 12 条 1 項
10 改正後施行規則 1 条 1 号の 2、10 条 1 項

まえ、定期的な報告に変更されました[11]。

Ⅱ. 薬機法等改正法案成立

2025 年 5 月 14 日、医薬品、医療機器等の品質、有効性及び安全性の確保等に関する法律等の一部を改正する法律（「改正薬機法」）が特段の修正なく成立しました。改正薬機法の要点については、本ニュースレター1 月号[12]で紹介したとおりですが、採決にあたっては衆参両院で附帯決議が採択されています[13]。

最も多くの附帯決議が採択されたのが、条件付き承認制度の見直しに関してです。今回の改正では、医療上特に必要性が高く、効能又は効果が合理的に予測できる医薬品等について、承認の取消し規定を設けたうえで、探索的試験の段階で条件付きで承認を与えることができるものとされました。これに関し、承認後に行う検証的臨床試験の内容及び臨床試験成績に関する資料を提出する期限等を可能な限り具体的に定め、正当な理由なく期限内に検証的臨床試験によって有効性及び安全性が確認できなかった場合には承認取り消し権限を適切に行使することの他、米国の迅速承認制度を教訓に適切な運用を図ること、市販後安全対策の内容を具体的に定めること、条件付き承認制度下での承認製品であることや承認条件等の明記・情報提供を行うこと、医薬品副作用救済制度による救済を行うこと等を求める附帯決議がなされました。

また、リアルワールドデータの利活用の観点から医薬品の承認申請時の資料に関する規定の文言が「臨床試験の試験成績に関する資料その他の資料」から「当該申請に係る医薬品、医薬部外品又は化粧品の品質、有効性及び安全性に関する資料として厚生労働省令で定める資料」に一般化されました。かかる改正に関しては、比較臨床試験が最も信頼性が高く、添付資料は原則として臨床試験の試験成績に関する資料である点に変わりがないことを確認するとともに、リアルワールドデータのみに基づく薬事承認は慎重に検討することが決議されています。

さらに、薬事に関する業務に責任を有する役員の変更命令が規定された点に関しては、事業者の経営権にも十分に配慮し、事業者が自律的に役員体制の見直しを行えるようにあらかじめ必要な指導を徹底すること、及び役員の変更命令を発出する場合の判断の考え方や、手順をあらかじめ公表することが決議されました。

その他、医薬品安定供給、革新的医薬品等実用化支援基金、零売規制、指定乱用防止医薬品の販売規制に関する事項も含め、のべ 34 の附帯決議が採択されました。附帯決議は法的な効力を持ちませんが[14]、政府はこれを尊重する一定の政治的な責任を負うとの見解もあり、今回の附帯決議も今後改正薬機法に基づく施行令・施行規則の内容又は行政通知等を通じて実務に影響を与えることも考えられますので、それらの動向が引き続き注目されます。

なお、当事務所では 2025 年 7 月 25 日に薬機法改正に関するセミナーを開催いたします。詳細については追って当事務所ウェブサイトで告知いたします。

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11 改正後施行規則 54 条 2 項 3 号、5 号

12 https://www.amt-law.com/asset/pdf/bulletins20_pdf/250131.pdf

13 第 217 回国会閣法 15 号　附帯決議　医薬品、医療機器等の品質、有効性及び安全性の確保等に関する法律等の一部を改正する法律案に対する附帯決議
衆議院
（https://www.shugiin.go.jp/internet/itdb_rchome.nsf/html/rchome/Futai/kourou5A89C5134451C2B749258C6E0004183E.htm）
参議院
(https://www.sangiin.go.jp/japanese/gianjoho/ketsugi/current/f069_051301.pdf)

14 東京地判平成 14(2002)年 9 月 27 日判時 11811 号 113 頁他

1. Enforcement of the Amended Clinical Trials Act, etc.

The Act Partially Amending the Act on the Safety of Regenerative Medicine, etc., and the Clinical Trials Act was promulgated on June 14, 2024, and will come into effect on May 31, 2025.[1] Accordingly, the Regulation for Enforcement of the Clinical Trials Act will also be amended and come into effect on the same day. This article will focus on changes in these amendments that are considered to have a significant impact on companies.

1.1 Application of the Clinical Trials Act on observational studies

Under the Pre-Amendment Clinical Trials Act, "clinical trials" that are subject to the application of the Clinical Trials Act are defined as research to clarify the efficacy or safety of pharmaceuticals in humans, excluding (i) research using medical information or samples obtained as a result of providing the most appropriate medical care for patients without controlling the presence and degree of inspections, medications or other diagnosis, or medical practice for treatment for research purposes (so-called observational studies), (ii) clinical trials conducted under the Pharmaceuticals and Medical Devices Act for marketing authorizations, (iii) post-marketing surveillance, etc., and (iv) research conducted to collect information on compliance with certification standards of medical devices.[2] Following the amendment, the phrase "including cases where, in the use of such pharmaceuticals for diagnosis, treatment, or prevention of human disease, or affecting the structure or function of the human body, additional inspections and other actions necessary to clarify the efficacy or safety of pharmaceuticals (limited to those specified by an Ordinance of the Ministry of Health, Labour and Welfare as imposing a significant burden on the person's mental or physical condition)" has been added to the definition of "clinical trials" and even so-called observational studies are subject to the Clinical Trials Act when such inspections that impose a significant burden on research subjects are conducted for research purposes.[3] Furthermore, the Post-Amendment Regulation for Enforcement provided that "acts that impose a significant burden on a person's mental or physical condition" will now include "inspections or other acts conducted on subjects of clinical trials, where the frequency of the occurrence of serious illness, disability or death, or infection or other events concerning the safety of clinical trials, or the degree of mental or physical suffering or burden is found to be considerably higher when such act is conducted than regular inspections or other acts"[4].

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1 The Clinical Trials Act and the Regulation for Enforcement of the Act on the Clinical Trials Act before the amendment are hereinafter referred to as the "**Pre-Amendment Clinical Trials Act**" and the "**Pre-Amendment Regulation for Enforcement**" respectively, and the Clinical Trials Act and the Regulation for Enforcement of the Clinical Trials Act after the amendment are hereinafter referred to as the "**Post-Amendment Clinical Trials Act**" and the "**Post-Amendment Regulation for Enforcement**" respectively.

2 Article 2, paragraph (1) of the Pre-Amendment Clinical Trials Act, and the items of Article 2 of the Pre-Amendment Regulation for Enforcement.

3 Article 2, paragraph (1) of the Post-Amendment Clinical Trials Act

4 Article 2-2 of the Post-Amendment Regulation for Enforcement

[The scope of coverage of Post-Amendment Clinical Trials Act]

Use of pharmaceutical products	Inspections, etc.	Applicability to the Clinical Trials Act
1. Use of pharmaceutical products **for research purposes**	(regardless of its contents)	**Applicable**
2. Use of pharmaceutical products **as provisions of usual medical care**	Cases where inspections, etc. which impose a significant burden on research subjects are conducted for research purposes in addition to usual medical care Example: Bone marrow aspiration, CT scan with contrast, etc. (Specified by Ordinance of the Ministry of Health, Labour and Welfare, etc., after hearing the opinion of the Health Science Council)	**Applicable**
	Cases where inspections, etc., other than the above are conducted in addition to usual medical care	**Not Applicable**
	Cases where only inspections, etc. are conducted within the scope necessary for usual medical care (inspections, etc. for research purposes are not conducted)	**Not Applicable**

(The Ministry of Health Labour and Welfare Website (https://www.mhlw.go.jp/content/10800000/001264187.pdf), translated by authors)

1.2 Scope of specified clinical trials on the use of off-label pharmaceuticals

Under the Post-Amendment Clinical Trials Act, "specified clinical trials" refer to clinical trials that (i) are conducted with research funds or other benefits provided by a manufacturer with marketing authorization for pharmaceuticals or a specially related person and/or (ii) use unauthorized pharmaceuticals or use pharmaceuticals for off-label purposes, and require compliance with clinical trial standards and submission of a trial plan.[5] In cases of off-label use, if the use of pharmaceuticals in the actual clinical trial slightly differs from the authorized dosage, administration, efficacy, and effect, it was uniformly considered to fall under the category of specified clinical trials. Therefore, there were opinions that the burden imposed on researchers is greater than the expected risk to research subjects. In response to such opinions, the amendment provides that if the risk to life and health of research subjects is equivalent to or less than the level associated with the authorized usage etc. of the pharmaceutical product, they are not considered as specified clinical trials[6].

In addition, the Post-Amendment Regulation for Enforcement provides that the following methods of use, etc., are low risk and do not fall under the category of specified clinical trials[7] (however, this excludes dosage or the like that have not been frequently used for medical care or causes hazards to public health and hygiene in Japan).

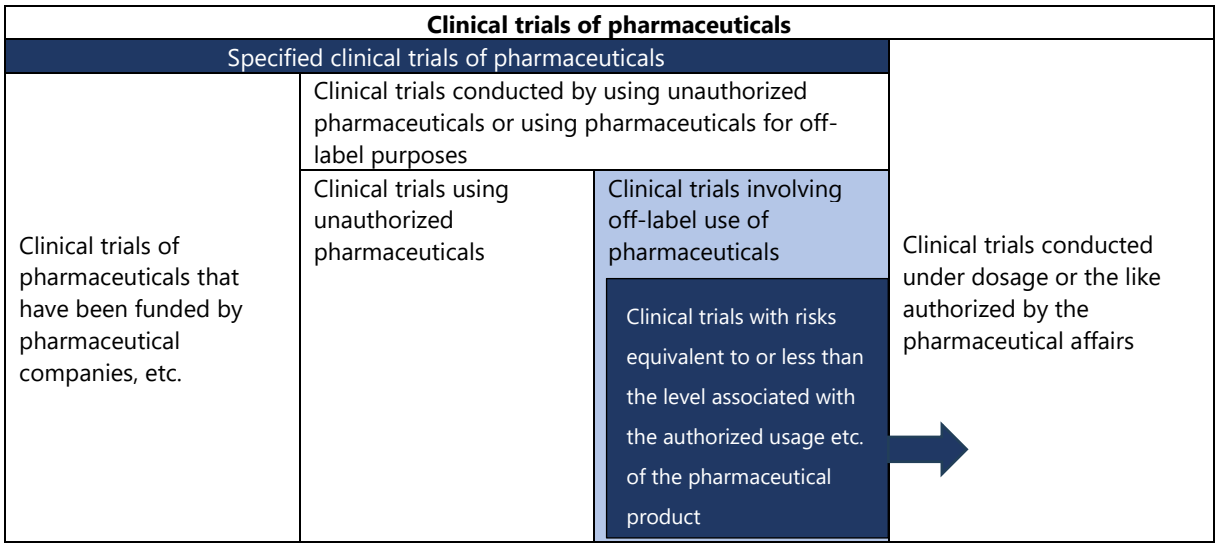
(i) Methods of use promoted by academic medical societies through the publication of guidelines for the implementation of appropriate medical care, etc. or similar measures.

5 Article 2, paragraph (2), Article 4, paragraph (2), and Article 5 of the Pre-Amendment Clinical Trials Act

6 Article 2, paragraph (2), item (ii) of the Post-Amendment Clinical Trials Act

7 Article 5 of the Post-Amendment Regulation for Enforcement

(ii) Methods of use where the efficacy, effect, and performance are within the scope of marketing authorization, and its efficacy and safety for research subjects are recognized.



(The Ministry of Health, Labour and Welfare Website (https://www.mhlw.go.jp/content/10808000/001354318.pdf), translated by authors)

1.3 Introduction of "general manager"

Under the Pre-Amendment Regulation for Enforcement, the responsibility for each action concerning the conduct of research was entrusted to the principal investigator[8], and in the case of multicenter joint research, the principal investigator assigned to each participating medical institution handled certain matters, such as audits, monitoring, and disease reporting. It has been pointed out that this was cumbersome, and that the determination of causality and each procedure were different even within the same research. Therefore, based on international trends, the amendment newly established a general manager to supervise the overall clinical trial and to take responsibility for the planning and operation of research, which is a part of the responsibility that had previously been assigned to the principal investigator. In the case of multicenter joint research, one general manager is to be appointed for a single clinical trial[9], and corporations and organizations may also serve as the general manager[10].

1.4 Review of the scope of disease reporting

Under the Pre-Amendment Regulation for Enforcement, periodic reporting (about once a year) was required for cases where pharmaceuticals were unauthorized or were used for off-label purposes, whereas reporting within 30 days was required for cases involving authorized pharmaceuticals. Therefore, it has been pointed out that the expected risks and reporting due dates are contradictory. Under the amendment, the deadline for reporting known serious diseases to the CRB (Certified Clinical Research Review Board) in clinical trials of unauthorized or off-label pharmaceuticals is, in principle,

8 Article 14 of the Pre-Amendment Clinical Trials Act and Article 17, 18, etc. of the Regulation for Enforcement of the same Act

9 Article 12, paragraph (1) of the Post-Amendment Regulation for Enforcement

10 Article 1, item (i)-2 and Article 10, paragraph (1) of the Post-Amendment Regulation for Enforcement

within 30 days. On the other hand, the reporting of known adverse reactions, including serious reactions, occurring in clinical trials of authorized pharmaceuticals is changed to periodic reporting, since such events can also occur in usual medical care. [11]

2. Enactment of the revision bill to the Pharmaceuticals and Medical Devices Act, etc.

On May 14, 2025, the Act Partially Amending the Act on Securing Quality, Efficacy, and Safety of Products Including Pharmaceuticals and Medical Devices, and other Acts (the "Amended Pharmaceuticals Act") was enacted without any particular modification. The main points of the Amended Pharmaceuticals Act were previously introduced in the January issue of this Newsletter, but supplementary resolutions were adopted by both houses of the Diet in preparation for the vote.[12] Most of the adopted supplementary resolutions were in relation to the review of the conditional marketing authorization system. The amendment allows conditional marketing authorization to be granted at the stage of exploratory trials with provisions for the rescindment of marketing authorization for pharmaceuticals that fulfill particularly high medical needs and whose efficacy or effect can be reasonably predicted. In this regard, supplementary resolutions were adopted to provide that the deadline for submission of data on the content of the confirmatory clinical studies after marketing authorization shall be stipulated as specifically as possible, and the authority to rescind marketing authorization shall be appropriately exercised in cases where efficacy and safety cannot be confirmed by confirmatory clinical studies within the deadline without justifiable reasons. The supplemental resolutions were also adopted to ensure appropriate operation based on lessons learned from the US Accelerated Approval Program, to provide details on post-marketing safety measures, to specify and provide information on products that are authorized under the conditional marketing authorization system and marketing authorization conditions, etc., and to provide relief through the Relief System for Sufferers from Adverse Drug Reactions.

In addition, from the viewpoint of the utilization of real world data, the phrase in the provision concerning the data required at the time of application for marketing authorization for pharmaceuticals has been generalized from "data concerning the results of clinical studies and other pertinent data" to "data provided by the Order of the Ministry of Health, Labour and Welfare as data concerning the quality, efficacy, and the safety of pharmaceuticals, quasi-pharmaceutical products or cosmetics pertaining to the application." Regarding such amendment, it was resolved to confirm that there is no change in the fact that controlled clinical studies are the most reliable, that supporting data shall, in principle, be data on the results of clinical studies, and that marketing authorization based solely on real world data should be carefully considered.

Moreover, regarding the provision of orders to change officers responsible for operations related to the

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11 Article 54, paragraph (2), item (iii) and item (v) of the Post-Amendment Regulation for Enforcement

12 Supplementary Resolutions to the Bill for Partial Amendment of the Act on Securing Quality, Efficacy and Safety of Pharmaceuticals and Medical Devices, etc. —Bill No. 15 of the 217th Diet Session
House of Representatives:
(https://www.shugiin.go.jp/internet/itdb_rchome.nsf/html/rchome/Futai/kourou5A89C5134451C2B749258C6E0004183E.htm)
House of Councillors:
(https://www.sangiin.go.jp/japanese/gianjoho/ketsugi/current/f069_051301.pdf)

pharmaceutical affairs, it was resolved that necessary guidance should be thoroughly provided in advance so that a business operator can review its constitution of officers autonomously while giving sufficient consideration to the management rights of the business operator, and that the considerations and procedures for making judgments for issuing an order to change officers should be publicly announced in advance.

Furthermore, a total of 34 supplementary resolutions were adopted, including those concerning the stable supply of pharmaceuticals, the practical application support fund for innovative pharmaceuticals, regulations on sales of non-prescription pharmaceuticals, and sales restrictions on designated anti-abuse pharmaceuticals. Although the supplementary resolutions have no legal effect[13], there is a view that the government bears certain political responsibility to respect them. The supplementary resolutions may also have an impact on practical operations through the content of the enforcement orders and enforcement regulations based on the Amended Pharmaceuticals Act or through administrative notifications, etc., and therefore, close monitoring of these developments remains important.

We are preparing a seminar regarding the amendments to the Pharmaceuticals and Medical Devices Act. Details of the seminar will be announced on our website shortly.

13 Tokyo District Court Judgment, September 27, 2002 (*Hanreijihou* No. 1181, p. 113)

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■ 本ニュースレターの執筆者は、以下のとおりです。

弁護士 近藤 純一 (junichi.kondo_grp@amt-law.com)
弁護士 淺井 茉里菜 (marina.asai@amt-law.com)
弁護士 藏野 舞 (mai.kurano@amt-law.com)
弁護士 横田 瑛弓 (emi.yokota@amt-law.com)

Authors:
Junichi Kondo (junichi.kondo_grp@amt-law.com)
Marina Asai (marina.asai@amt-law.com)
Mai Kurano (mai.kurano@amt-law.com)
Eimi Yokota (emi.yokota@amt-law.com)

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